UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
WASHINGTON, DC 20549

FORM 10-Q

(Mark One)

☒ **QUARTERLY REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934**

For the quarterly period ended March 31, 2016

or

☐ **TRANSITION REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934**

Commission File Number: 001-37566

Mirna Therapeutics, Inc.
(Exact name of registrant as specified in its charter)

Delaware	**26-1824804**
(State or other jurisdiction of incorporation or organization)	**(I.R.S. Employer Identification No.)**

2150 Woodward Street, Suite 100
Austin, TX **(Address of principal executive offices)**

78744
(Zip Code)

(512) 901-0900
(Registrant's telephone number, including area code)

Indicate by check mark whether the registrant (1) has filed all reports required to be filed by Section 13 or 15(d) of the Securities Exchange Act of 1934 during the preceding 12 months (or for such shorter period that the registrant was required to file such reports), and (2) has been subject to such filing requirements for the past 90 days. Yes ☒ No ☐

Indicate by check mark whether the registrant has submitted electronically and posted on its corporate Web site, if any, every Interactive Data File required to be submitted and posted pursuant to Rule 405 of Regulation S-T (§232.405 of this chapter) during the preceding 12 months (or for such shorter period that the registrant was required to submit and post such files). Yes ☒ No ☐

Indicate by check mark whether the registrant is a large accelerated filer, an accelerated filer, a non-accelerated filer, or a smaller reporting company. See definitions of "large accelerated filer," "accelerated filer" and "smaller reporting company" in Rule 12b-2 of the Exchange Act.

Large accelerated filer	☐	Accelerated filer	☐
Non-accelerated filer	☒ (Do not check if a smaller reporting company)	Smaller reporting company	☐

Indicate by check mark whether the registrant is a shell company (as defined in Rule 12b-2 of the Exchange Act). Yes ☐ No ☒

As of May 10, 2016 there were 20,830,555 shares of the registrant's common stock, par value $0.001 per share, outstanding.

Mirna Therapeutics, Inc.

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PART I—FINANCIAL INFORMATION

Item 1.

Condensed Financial Statements
Mirna Therapeutics, Inc.
Condensed Balance Sheets

(in thousands, except share and per share data)

	March 31, 2016	December 31, 2015
Assets	(unaudited)	
Current Assets:		
Cash and cash equivalents	$ 52,875	$ 89,713
Marketable securities	27,713	—
Grant reimbursement and other receivables	168	36
Prepaid expenses and other current assets	853	793
Total current assets	81,609	90,542
Property and equipment, net	516	375
Total assets	$ 82,125	$ 90,917
Liabilities and Stockholders' Equity		
Current Liabilities:		
Accounts payable	$ 1,239	$ 3,687
Accrued expenses	1,985	2,214
Total liabilities	3,224	5,901
Commitments and contingencies		
Stockholders' Equity (Deficit):		
Preferred stock, $0.001 par value, 5,000,000 shares authorized at March 31, 2016 and December 31, 2015; 0 shares outstanding at March 31, 2016 and December 31, 2015	—	—
Common stock, $0.001 par value; 250,000,000 shares authorized at March 31, 2016 and December 31, 2015; 20,830,555 shares issued and outstanding at March 31, 2016 and December 31, 2015	21	21
Additional paid in capital	161,965	161,518
Accumulated other comprehensive income	9	—
Accumulated deficit	(83,094)	(76,523)
Total stockholders' equity	78,901	85,016
Total liabilities and stockholders' equity	$ 82,125	$ 90,917

The accompanying notes are an integral part of these condensed financial statements.

Mirna Therapeutics, Inc.
Condensed Statements of Operations and Comprehensive Loss (Unaudited)

(in thousands, except share and per share data)

	Three Months Ended March 31,	
	2016	2015
Operating expenses:		
Research and development	$ 4,523	$ 3,402
General and administrative	2,130	877
Total operating expenses	6,653	4,279
Other income:		
Interest income	82	—
Total other income	82	—
Net loss	$ (6,571)	$ (4,279)
Less: Accretion and dividends on convertible preferred stock	—	(1,118)
Net loss attributable to common stockholders	$ (6,571)	$ (5,397)
Other comprehensive income:		
Unrealized gain on available for sale securities, net of tax	9	—
Total Other Comprehensive (Loss)	(6,562)	—
Net loss per share attributable to common stockholders—basic and diluted	$ (0.32)	$ (60.99)
Common shares used to compute basic and diluted net loss per share attributable to common stockholders	20,830,555	88,484

The accompanying notes are an integral part of these condensed financial statements.

Mirna Therapeutics, Inc.
Condensed Statements of Cash Flows (Unaudited)

(in thousands)

	Three Months Ended March 31,	
	2016	**2015**
Operating activities		
Net loss	$ (6,571)	$ (4,279)
Adjustment to reconcile net loss to net cash used in operating activities:		
Depreciation and amortization	19	12
Stock-based compensation	447	134
Net amortization of premium/ discounts on marketable securities	19	—
Changes in operating assets and liabilities:		
Grant reimbursement and other receivables	(132)	(26)
Prepaid expenses and other current assets	(60)	16
Deferred financing costs	—	92
Accounts payable	(2,448)	199
Accrued expenses	(247)	(698)
Net cash used in operating activities	(8,973)	(4,550)
Investing activities		
Purchases of marketable securities	(27,722)	—
Purchase of property and equipment	(143)	(5)
Net cash used in investing activities	(27,865)	(5)
Financing activities		
Proceeds from issuance of convertible preferred stock, net of issuance costs	—	35,346
Proceeds from the exercise of stock options	—	20
Cash provided by financing activities	—	35,366
Net increase (decrease) in cash and cash equivalents	(36,838)	30,811
Cash and cash equivalents at beginning of period	89,713	9,319
Cash and cash equivalents at end of period	$ 52,875	$ 40,130

The accompanying notes are an integral part of these condensed financial statements.

Mirna Therapeutics, Inc.

Notes to Condensed Financial Statements (Unaudited)

1. Nature of Business and Basis of Presentation

Nature of business

Mirna Therapeutics, Inc. ("Mirna" or "the Company") is a clinical stage biopharmaceutical company developing a broad pipeline of microRNA-based oncology therapeutics. The Company was incorporated in Delaware in December 2007 as a wholly-owned subsidiary of Asuragen, Inc. ("Asuragen") and was spun out to existing Asuragen stockholders in December 2009. The Company is located in Austin, Texas.

In October 2015, the Company sold 6,250,000 shares of common stock, $0.001 par value per share, in an underwritten public offering (the "IPO") and 2,395,010 shares of common stock in a concurrent private placement, with both offerings at a price of $7.00 per share. The underwriters of the IPO purchased an additional 704,962 shares of common stock pursuant to their option to purchase additional shares. The Company's aggregate net proceeds from the IPO were $43.7 million, after deducting the transaction offering costs and the underwriting discounts incurred. The Company also received net proceeds of $16.7 million after deducting the offering transaction costs from the concurrent private placement.

The Company continues to be subject to a number of risks common to companies in similar stages of development. Principal among these risks are the uncertainties of technological innovations, dependence on key individuals, development of the same or similar technological innovations by the Company's competitors and protection of proprietary technology. The Company's ability to fund its planned clinical operations, including completion of its planned trials, is expected to depend on the amount and timing of cash receipts from future collaboration or product sales and/or financing transactions. The Company believes that its cash and cash equivalents and marketable securities of $80.6 million at March 31, 2016 will enable the Company to maintain its current and planned operations for the foreseeable future.

Basis of presentation

The accompanying interim condensed financial statements as of March 31, 2016 and for the three months ended March 31, 2016 and 2015, and the related interim information contained within the notes to the financial statements, are unaudited. The unaudited interim condensed financial statements have been prepared in accordance with generally accepted accounting principles for interim financial information and on the same basis as the audited financial statements. In the opinion of management, the accompanying unaudited interim condensed financial statements contain all adjustments which include only normal recurring adjustments necessary to state fairly the Company's financial position as of March 31, 2016, and the results of its operations and cash flows for the three months ended March 31, 2016 and 2015. Such adjustments are of a normal and recurring nature. The interim financial data as of March 31, 2016 is not necessarily indicative of the results to be expected for the year ending December 31, 2016, or for any future period.

The accompanying condensed financial statements and related financial information should be read in conjunction with the audited financial statements and related notes thereto for the year ended December 31, 2015 included in the Company's Form 10-K, most recently filed with the Securities and Exchange Commission on March 30, 2016.

2. Summary of Significant Accounting Policies

Use of estimates

The preparation of financial statements in conformity with U.S. generally accepted accounting principles requires the Company's management to make estimates and assumptions that affect the amounts reported in the financial statements and accompanying notes. Actual results could differ from those estimates.

Research and development costs

Research and development costs are expensed as incurred. Research and development costs include salaries and personnel-related costs, consulting fees, fees paid for contract research services, the costs of laboratory equipment and facilities, development of intellectual property, license fees and other external costs. The Company accounts for government grants as a reduction of research and development expenses. Government grants are recorded at the time the related research and development costs have been incurred by the Company and, accordingly, become eligible for reimbursement. The Company accrues for government grants that have been earned but not yet received.

Nonrefundable advance payments for goods or services to be received in the future for use in research and development activities are deferred and capitalized. The capitalized amounts are expensed as the related goods are delivered or the services are performed.

Clinical Trial and Pre-Clinical Study Accruals

The Company estimates pre-clinical study and clinical trial expenses pursuant to contracts with research institutions and contract research organizations that conduct and manage preclinical studies and clinical trials on the Company's behalf. These estimates are based on the level of service performed and the underlying agreement. Further, the Company accrues expenses related to clinical trials based on the level of patient enrollment and other activities according to the related agreements. The Company monitors patient enrollment levels and other activities to the extent reasonably possible and adjusts estimates accordingly.

Stock-based compensation

The Company accounts for its stock-based compensation awards in accordance with ASC Topic 718, *Compensation—Stock Compensation* ("ASC 718"). ASC 718 requires all stock-based payments to employees, including grants of employee stock options, to be recognized in the statements of operations based on their grant date fair values. For stock options granted to employees and to members of the board of directors for their services on the board of directors, the Company estimates the grant date fair value of each option award using the Black-Scholes option-pricing model. The use of the Black-Scholes option-pricing model requires management to make assumptions with respect to the expected term of the option, the expected volatility of the common stock consistent with the expected life of the option, risk-free interest rates and expected dividend yields of the common stock. For awards subject to service-based vesting conditions, the Company recognizes stock-based compensation expense, net of estimated forfeitures, equal to the grant date fair value of stock options on a straight-line basis over the requisite service period.

Fair value measurements

The Company records money market funds at fair value. ASC Topic 820, *Fair Value Measurements and Disclosures*, establishes a fair value hierarchy for those instruments measured at fair value that distinguishes between assumptions based on market data (observable inputs) and the Company's own assumptions (unobservable inputs). The hierarchy consists of three levels:

- Level 1 – Unadjusted prices in active markets for identical assets or liabilities.
- Level 2 – Quoted prices for similar assets and liabilities in active markets, quoted prices in markets that are not active, or inputs which are observable, either directly or indirectly, for substantially the full term of the asset or liability.
- Level 3 – Unobservable inputs that reflect the Company's own assumptions about the assumptions market participants would use in pricing the asset or liability in which there is little, if any, market activity for the asset or liability at the measurement date.

The carrying amounts reflected in the balance sheets for cash, prepaid expenses and other current assets, accounts payable, and accrued expenses approximate their fair values at March 31, 2016 and December 31, 2015, due to their short-term nature.

There have been no changes to the valuation methods for the three months ended March 31, 2016 and 2015. The Company evaluates transfers between levels at the end of each reporting period. There were no transfers of assets or liabilities between Level 1, Level 2 or Level 3 during the three months ended March 31, 2016 or 2015.

Marketable Securities

The Company classifies marketable securities with a remaining maturity when purchased of greater than three months as available-for-sale. Marketable securities with a remaining maturity date greater than one year are classified as non-current. Available-for-sale securities are maintained by an investment manager and may consist of U.S. Treasury securities and government agency securities and corporate debt securities. Available-for-sale securities are carried at fair value with the unrealized gains and losses included in other comprehensive income (loss) as a component of stockholders' equity until realized. Any premium or discount arising at purchase is amortized and/or accreted to interest income and/or expense over the life of the instrument. Realized gains and losses are determined using the specific identification method and are included in other income (expense).

If any adjustment to fair value reflects a decline in value of the investment, the Company considers all available evidence to evaluate the extent to which the decline is "other-than-temporary" and, if so, mark the investment to market through a charge to the Company's statement of operations and comprehensive loss.

Comprehensive loss

Comprehensive loss is composed of net loss and other comprehensive income or loss. Other comprehensive income consists of unrealized gains on marketable securities.

Recent Accounting Pronouncements

In March 2016, the Financial Accounting Standards Board (FASB) issued ASU 2016-09, *Compensation - Stock Compensation (Topic 718): Improvements to Employee Share Based Payment Accounting* ("ASU 2016-09") as part of the FASB simplification initiative. The new standard provides for changes to accounting for stock compensation including 1) excess tax benefits and tax deficiencies related to share based payment awards will be recognized as income tax expense in the reporting period in which they occur; 2) excess tax benefits will be classified as an operating activity in the statement of cash flow; 3) the option to elect to estimate forfeitures or account for them when they occur; and 4) increase tax withholding requirements threshold to qualify for equity classification. The ASU is effective for public companies for annual periods, and interim periods within those annual periods, beginning after December 15, 2016, and early adoption is permitted. The Company is currently evaluating the impact that ASU 2016-09 will have on the unaudited condensed financial statements.

In February 2016, the FASB issued ASU 2016-02, *Leases (Topic 842)*. The new standard requires the recognition of assets and liabilities arising from lease transactions on the balance sheet and the disclosure of key information about leasing arrangements. Accordingly, a lessee will recognize a lease asset for its right to use the underlying asset and a lease liability for the corresponding lease obligation. Both the asset and liability will initially be measured at the present value of the future minimum lease payments over the lease term. Subsequent measurement, including the presentation of expenses and cash flows, will depend on the classification of the lease as either a finance or

an operating lease. Initial costs directly attributable to negotiating and arranging the lease will be included in the asset. For leases with a term of twelve months or less, a lessee can make an accounting policy election by class of underlying asset to not recognize an asset and corresponding liability. Lessees will also be required to provide additional qualitative and quantitative disclosures regarding the amount, timing and uncertainty of cash flows arising from leases. These disclosures are intended to supplement the amounts recorded in the financial statements and provide additional information about the nature of an organization's leasing activities. The new standard is effective for fiscal years beginning after December 15, 2018, and interim periods within those years, with early adoption permitted. In transition, lessees are required to recognize and measure leases at the beginning of the earliest period presented using a modified retrospective approach. The transition guidance also provides specific guidance for sale and leaseback transactions, build-to-suit leases and amounts previously recognized in accordance with the business combinations guidance for leases. We are currently evaluating our expected adoption method and the impact of this new standard on the unaudited condensed financial statements.

3. Marketable Securities

The following table summarizes the available-for-sale securities held at March 31, 2016 (in thousands):

March 31, 2016	Amortized Cost	Unrealized Gains	Unrealized Losses	Fair Value
U.S. government agency securities and treasuries	$ 19,046	$ 6	$ —	$ 19,052
Corporate debt securities	8,658	3	—	8,661
Total available-for-sale securities	$ 27,704	$ 9	$ —	$ 27,713

The Company did not have available for sale securities at December 31, 2015. No available- for- sale securities held as of March 31, 2016 had remaining maturities greater than one year.

4. Fair Value Measurements

The following table sets forth the Company's assets that are measured at fair value on a recurring basis as of March 31, 2016 and December 31, 2015 (in thousands):

	Total	Quoted prices in active markets (Level 1)	Significant other observable inputs (Level 2)	Significant unobservable inputs (Level 3)
March 31, 2016				
Assets:				
Money Market Funds	$ 46,118	$ 46,118	$ —	$ —
US government agency securities and treasuries	6,757	—	6,757	—
Total cash and cash equivalents	52,875	46,118	6,757	—
Marketable securities:				
U.S. government agency securities and treasuries	19,052	—	19,052	—
Corporate debt securities	8,661	—	8,661	—
Total marketable securities	27,713	—	27,713	—
Total assets	$ 80,588	$ 46,118	$ 34,470	$ —
December 31, 2015				
Assets:				
Money Market Funds	89,713	89,713	—	—
Total Assets	$ 89,713	$ 89,713	$ —	$ —

Cash and cash equivalents
The Company considers all highly liquid securities with original final maturities of three months or less from the date of purchase to be cash equivalents. As of March 31, 2016 and December 31, 2015, cash and cash equivalents are comprised of money market accounts and U.S. government agency securities.

Marketable securities
The amortized cost of available-for-sale securities is adjusted for amortization of premiums and accretion of discounts to maturity. At March 31, 2016 and December 31, 2015, the balance in the Company's accumulated other comprehensive income was composed solely of activity related to the Company's available-for-sale marketable securities. There were no realized gains or losses recognized on the sale or maturity of available-for-sale securities during the three months ended March 31, 2016, and, as a result, the Company did not reclassify any amounts of accumulated other comprehensive income for the same period.

There were not available for sale securities held by the Company in an unrealized loss position at March 31, 2016. The Company has the intent and ability to hold such securities until recovery. The Company determined that there was no material changes in the credit risk of the above investments. As a result, the Company determined it did not hold any investments with an other- than- temporary impairment as of March 31, 2016 and December 31, 2015.

5. Property and Equipment

Property and equipment consisted of the following (in thousands):

	March 31, 2016	December 31, 2015
Machinery, computers and equipment	$ 847	$ 687
Leasehold improvements	18	18
Accumulated depreciation	(349)	(330)
	$ 516	$ 375

Depreciation expense was approximately $19,000 and $12,000 for the three months ended March 31, 2016 and 2015, respectively.

6. Common Stock

The voting, dividend and liquidation rights of holders of shares of common stock are subject to and qualified by the rights, powers and preferences of the holders of shares of convertible preferred stock. The Company's common stock has the following characteristics:

- The holders of shares of common stock are entitled to one vote for each share of common stock held at all meetings of stockholders and written actions in lieu of meetings.
- The holders of shares of common stock are entitled to receive dividends, if and when declared by the Company's board of directors.

Cash dividends may not be declared or paid to holders of common stock until paid on each series of outstanding convertible preferred stock in accordance with their respective terms. As of March 31, 2016 and December 31, 2015, no cash dividends have been declared.

Offerings

In September 2015, the Company entered into a new grant contract with Cancer Prevention and Research Institute of Texas ("CPRIT"), in connection with an award of approximately $16.8 million. The 2015 award was in the form of an agreement by CPRIT to purchase $16.8 million of shares of common stock of the Company in a private placement concurrent with the initial public offering of the Company's common stock. On October 5, 2015, CPRIT purchased 2,395,010 shares of the Company's common stock at $7.00 per share. Net proceeds from the private placement, after related transaction offering costs, were approximately $16.6 million.

In October 2015, the Company issued 6.25 million shares of common stock in an underwritten public offering, with a price of $7.00 per share. The underwriters purchased an additional 704,962 shares of common stock pursuant to their option to purchase additional shares. The Company received aggregate net proceeds of approximately $43.7 million in the public offering, after deducting the underwriting discounts and commissions and estimated offering expenses payable by the Company.

7. Stock Option Plans

2008 Long Term Incentive Plan

During 2008, the Company adopted the 2008 Long Term Incentive Plan, which allows for incentive stock options for its employees and nonqualified stock options (inclusive of restricted stock units and stock appreciation rights) (the "2008 Plan") for employees and nonemployees under which an aggregate of 330,582 stock options and stock purchase rights may be granted. In December 2013, the total amount available for grant under the 2008 Plan was

increased by 224,200 to 554,782. In March 2014, the Company's board of directors approved an increase of 115,153 shares available for grant pursuant to the 2008 Plan to 669,935. In March 2015, the total amount of available to grant under the 2008 Plan was increased in conjunction with the Company's offering of Series D preferred stock by 391,650 shares to 1,061,585. Options under the 2008 Plan have a maximum life of 10 years. Options vest at various intervals, as determined by the Company's board of directors at the date of grant.

2015 Equity Incentive Plan

In August 2015, the Company's board of directors approved the 2015 Equity Incentive Award Plan, (the "2015 Plan"), which was effective in connection with the pricing of the IPO on September 30, 2015. The 2015 Plan provides for the granting of a variety of stock-based compensation awards, including stock options, stock appreciation rights, or SARs, restricted stock awards, restricted stock unit awards, deferred stock awards, dividend equivalent awards, stock payment awards, performance awards and other stock-based awards. The 2015 Plan is the successor to the 2008 Plan and the 800,478 options outstanding in the 2008 Plan at March 31, 2016 may be transferred to the 2015 Plan if awards thereunder terminate, expire or lapse for any reason without the delivery of shares to the holder thereof. Under the 2015 Plan, 1,671,800 shares of the Company's common stock were initially authorized and reserved for issuance. In March 2016, the Company's board of directors approved an increase of 1,041,527 shares available for grant pursuant to the 2015 Plan. Accordingly, a total of 3,513,805 shares have been authorized and reserved for issuance under the 2015 Plan at March 31, 2016.

2015 Employee Stock Purchase Plan

In August 2015, the Company's board of directors approved the 2015 Employee Stock Purchase Plan (the "ESPP"), which was effective in connection with the pricing of the IPO on September 30, 2015. The ESPP allows eligible employees to purchase shares of the Company's common stock at a discount through payroll deductions of up to 15% of their eligible compensation, subject to any plan limitations. The ESPP generally provides for set offering periods, and at the end of each offering period, employees are able to purchase shares at 85% of the lower of the fair market value of the Company's common stock on the first trading day of the offering period or on the last trading day of the offering period. There were no sales under the ESPP as of March 31, 2016. Shares available for future purchase under the ESPP were 375,485 at March 31, 2016.

Stock Option Activity

The Company's stock option activity for the three months ended March 31, 2016 was as follows:

	Number of Shares	Weighted-Average Exercise Price	Weighted-Average Contractual Life (years)
Outstanding at December 31, 2015	1,529,459	6.29	9.00
Granted	386,250	4.37	
Exercised	—	—	
Forfeited/canceled	—	—	
Outstanding at March 31, 2016	1,915,709	$ 5.90	8.99
Options exercisable at March 31, 2016	420,417	$ 4.77	7.52

Stock Compensation Expense

Total stock- based compensation expense for the three months ended March 31, 2016 was allocated as follows in the statements of comprehensive loss (in thousands):

	Three Months Ended March 31,	
	2016	2015
Research and development expense	$ 171	$ 32
General and administrative expense	276	102
Total stock based compensation	$ 447	$ 134

As of March 31, 2016 there was approximately $5.9 million of unrecognized compensation cost related to the stock options granted under the 2015 Plan, which is expected to be amortized over a weighted average period of 3.2 years. There were no restricted stock units or stock appreciation rights granted under the 2015 Plan as of March 31, 2016.

8. Income Taxes

The Company recorded no provision for income taxes as of March 31, 2016 due to reported net losses since inception.

During the three months ended March 31, 2016 and 2015, the Company had no interest and penalties related to income taxes.

Deferred income taxes reflect the net tax effects of temporary differences between the carrying amount of assets and liabilities for financial reporting purposes and the amounts used for income tax purposes. The Company has established a valuation allowance due to uncertainties regarding the realization of deferred tax assets based upon the Company's lack of earnings history. The Company files income tax returns in the U.S. federal and Texas jurisdictions. The statute of limitations for assessment by the Internal Revenue Service ("IRS") is open for tax years ending December 31, 2014, 2013, 2012 and 2010, although carryforward attributes that were generated for tax years prior to 2011 may still be adjusted upon examination by the IRS if they either have been, or will be, used in a future period. The 2010 and subsequent tax years remain open and subject to examination by the State of Texas. There are currently no federal or state income tax audits in progress.

9. Shared Services Agreement with Asuragen

On November 3, 2009, the Company entered into an agreement with Asuragen under which Asuragen shares space with and provides services to the Company in support of the Company's business. Such services have included human resources, finance and accounting, information technology, purchasing, shipping and receiving, equipment use, and various facility expenses. The Company pays Asuragen a monthly service fee for the services provided by Asuragen to the Company, which does not include direct charges incurred by Asuragen on behalf of the Company. The Company paid Asuragen approximately $94,000 and $98,000 for the three months ended March 31, 2016 and 2015, respectively.

On October 31, 2014, the Company entered into a sublease agreement with Asuragen for use of office, laboratory and shared space. Total rent expense was $22,200 for the first three months of 2016. Both the lease and the shared service agreements expire on August 31, 2016.

10. License agreements

Rosetta Genomics Ltd.

In December 2015, the Company entered into a Patent License Agreement (the "License Agreement") with Rosetta Genomics Ltd. ("Rosetta"), licensing to the Company certain patents owned or controlled by Rosetta as specified in the License Agreement. Under the License Agreement, Rosetta has granted the Company a non-assignable, non- transferable, worldwide license for certain patents in connection with the development and commercialization of products that relate to the tumor suppressor microRNA MIR-34 ("Products"). This license is exclusive with respect to Products that relate to MRX34, the Company's lead product candidate, and non-exclusive for products that are not related.

Under the License Agreement, the Company paid Rosetta an up-front, non- refundable payment of $1.6 million, which was recorded as an expense within research and development in 2015 and subsequently paid in January 2016. The Company is obligated to pay low single- digit royalties on net sales of Products, as well as royalties on sublicense revenues. Certain development and regulatory milestone payments totaling $3 million may also be payable in connection with specified types of Products, upon the achievement of certain development and/ or regulatory milestone events.

Marina Biotech, Inc.

In December 2011, the Company entered into a licensing agreement with Marina, pursuant to which Marina granted to the Company a license to liposomal delivery technology, NOV340, known under the brand name "SMARTICLES," to develop and commercialize drug products incorporating Marina's delivery system exclusively in combination with the Company's lead therapeutic product, MRX34. In December 2013, the license agreement was amended to include three additional specific mimics selected by the Company to use with SMARTICLES on an exclusive basis, and in May 2015, the license agreement was further amended to reduce the amount of a specific milestone payment and to provide for the prepayment of such milestone payment. In August 2015, the Company also entered into a side letter to the license agreement, under which it exercised its right to select an additional specific microRNA, in exchange for the payment of a specified selection fee payment.

The Company has cumulatively paid Marina approximately $2.1 million through December 31, 2015 in up-front and milestone payments and as consideration for the inclusion within the license of four additional microRNA compounds. As the Company progresses with respect to development and commercialization of its products, the Company will be required to make payments to Marina based upon the achievement of certain development and regulatory milestones, totaling up to $6 million in the aggregate for each licensed product. The Company has agreed to pay up to an additional $4 million per licensed product upon the achievement of certain regulatory milestones for a specified number of additional indications, leading to a maximum cap on all milestone payments of $10 million per product. The exception to this is for the Company's lead therapeutic product, MRX34, where the aggregate of all remaining development and regulatory milestone payments due to Marina, including for all additional indications, is $4.0 million.

In addition to milestone payments, the Company will be required to pay low single digit royalties on net sales of licensed products other than MRX34, subject to customary reductions and offsets. As a result of the Company's 2013 amendment to the agreement with Marina, the Company is no longer required to pay a royalty to Marina with respect to sales of the Company's lead therapeutic product, MRX34. If the Company sublicenses its rights under the license from Marina, for each optioned microRNA compound covered by such sublicense the Company is required to pay a specified lump-sum payment representing the remainder of the selection fee for the inclusion of such microRNA compound within the scope of the license agreement, as well as a portion of any revenue the Company receives from such sublicensees at a tiered percentage between the very low single digits and the mid-teens, depending on the circumstances in which the sublicense is entered into.

Yale University

In 2006, Asuragen entered into an exclusive license agreement with Yale University ("Yale") under certain patent rights relating to microRNAs arising from the laboratory of Dr. Frank Slack. This agreement was assigned to the Company by Asuragen in connection with the Company's acquisition of certain assets, including patent rights, in 2009. In February 2014, the Company as successor-in-interest to Asuragen, amended and restated the exclusive license

agreement. Some of the patent filings in the Company's intellectual property portfolio that are licensed to the Company by Asuragen are also included in the patents licensed under the agreement with Yale. The Company will be required to pay royalties to Yale on net sales of licensed products that contain specified microRNAs, at a percentage ranging from the very low to the low single digits, subject to customary reductions and offsets. The Company will also be required to pay to Yale a portion of specified gross revenue that the Company receives from the Company's sublicensees at a percentage in the mid-single digits.

The Company will be required to make payments for achievement of certain development and regulatory milestones by products containing one specified microRNA and covered by the licensed patents, of up to $600,000 in the aggregate for each such product, subject to reduction in certain circumstances. In addition, the Company is required to pay an annual license maintenance fee and minimum annual royalties under certain circumstances.

11. Commitments and Contingencies

Shared Services Agreement

Pursuant to a shared services agreement and sublease with Asuragen (see Note 9), the Company has remaining commitments for payments through August 2016 under the shares services agreement and sublease of $197,510 and $37,000, respectively.

12. Net Loss Per Share Attributable to Common Stockholders

The following table summarizes the computation of basic and diluted net loss per share attributable to common stockholders of the Company (in thousands, except share and per share data):

	Three Months Ended March 31,	
	2016	2015
Net loss	$ (6,571)	$ (4,279)
Accretion of convertible preferred stock to redemption value	—	(422)
Accrued dividends on convertible preferred stock	—	(696)
Net loss attributable to common stockholders—basic and diluted	(6,571)	(5,397)
Weighted-average number of common shares—basic and diluted	20,830,555	88,484
Net loss per share attributable to common stockholders—basic and diluted	$ (0.32)	$ (60.99)

The following potentially dilutive securities outstanding, prior to the use of the treasury stock method or if-converted method, have been excluded from the computation of diluted weighted-average common shares outstanding, because including them would have had an anti-dilutive effect due to the losses reported.

	March 31,	
	2016	2015
Convertible preferred stock	—	9,472,340
Stock options	1,915,709	556,935
	1,915,709	10,029,275

As the Company incurred a net loss for the three months ended March 31, 2016 there is no income allocation required under the two-class method or dilution attributed to weighted-average shares outstanding in the computation of diluted loss per share attributable to common stockholders.

Item 2. Management's Discussion and Analysis of Financial Condition and Results of Operations

You should read the following management's discussion and analysis of our financial condition and results of operations in conjunction with our unaudited condensed financial statements and notes thereto included in Part I, Item 1 of this Quarterly Report on Form 10-Q and with our audited financial statements and notes thereto for the year ended December 31, 2015, filed with the U.S. Securities and Exchange Commission (SEC) on March 30, 2016.

Special note regarding forward-looking statements

This report contains forward-looking statements that involve risks and uncertainties. Our actual results could differ materially from those discussed in the forward-looking statements. The statements contained in this report that are not purely historical are forward-looking statements within the meaning of Section 27A of the Securities Act of 1933, as amended (the Securities Act), and Section 21E of the Securities Exchange Act of 1934, as amended (the Exchange Act). Forward-looking statements are often identified by the use of words such as, but not limited to, "anticipate," "believe," "can," "continue," "could," "estimate," "expect," "intend," "may," "plan," "project," "seek," "should," "strategy," "target," "will," "would" and similar expressions or variations intended to identify forward-looking statements. These statements are based on the beliefs and assumptions of our management based on information currently available to management. Such forward-looking statements are subject to risks, uncertainties and other important factors that could cause actual results and the timing of certain events to differ materially from future results expressed or implied by such forward-looking statements. Factors that could cause or contribute to such differences include, but are not limited to, those identified below and those discussed in the section titled "Risk Factors" included under Part II, Item 1A below. Furthermore, such forward-looking statements speak only as of the date of this report. Except as required by law, we undertake no obligation to update any forward-looking statements to reflect events or circumstances after the date of such statements.

Overview

We are a clinical-stage biopharmaceutical company developing a broad pipeline of microRNA-based oncology therapeutics. microRNAs are naturally occurring, short ribonucleic acid, or RNA, molecules, or oligonucleotides, that play a critical role in regulating key biological pathways. Misexpression of even a single microRNA can contribute to disease development and tumor suppressor microRNAs are commonly reduced in cancer. Our scientists and others at leading academic institutions have identified numerous tumor suppressor microRNAs that play key roles in preventing normal cells from becoming cancerous and facilitating proper cancer immunosurveillance. We are developing mimics of naturally occurring microRNAs that are designed to increase this tumor suppressor activity and aid appropriate anti- tumor immune response.

Our lead product candidate, MRX34, a mimic of naturally occurring microRNA-34 (miR-34) encapsulated in a liposomal nanoparticle formulation, is the first microRNA mimic to enter clinical development and has demonstrated clinical proof of concept as a single agent in our ongoing Phase 1 clinical trial. We plan to initiate, in the second half of 2016, an additional Phase 1b translational medicine trial to deepen our insights into the mechanism of action of miR-34 in melanoma patients and to define biomarkers that would aid in furthering the development of MRX34. We expect MRX34 to begin Phase 2 by the end of 2016. We are planning trials that will include patients with advanced malignant melanoma and patients with advanced renal cell carcinoma (RCC).

We believe that microRNA mimics may represent a new paradigm in cancer therapy and have the potential to create a new, important class of effective cancer drugs that can potentially be used alone or in combination with other cancer therapeutics. For the next wave of cancer therapies to produce a measurable improvement over current approaches, we believe it will need to yield drugs that can disrupt multiple oncogenic and immuno-oncology pathways. We believe the microRNA field represents a highly promising area for the development of these drugs.

We were incorporated in 2007 under the laws of Delaware and were maintained as a wholly-owned subsidiary of our former parent company, Asuragen, Inc., or Asuragen, until the end of 2009, when we became an independent entity.

Our operations have focused on developing our understanding of and capabilities in microRNA biology, identifying potential product candidates, undertaking preclinical studies, initiating and conducting a clinical trial, protecting and enhancing our intellectual property estate and providing general and administrative support for these activities. We have not generated any revenue from product sales and, to date, have funded our operations primarily through the private placements of convertible preferred stock, federal and state government grants and offerings of our common stock. From our inception through March 31, 2016, we have raised an aggregate of approximately $167.3 million to fund our operations, of which approximately $89.9 million was from the issuance of preferred stock for cash and assets, $48.7 million from a public offering of our common stock, $16.8 million from a private placement of our common stock and $11.9 million was from federal and state grants.

Since our inception, we have incurred significant operating losses. Our net loss was $6.6 million for the three months ended March 31, 2016. At March 31, 2016, we had an accumulated deficit of $83.1 million. We expect to continue to incur significant expenses and operating losses over the next several years. Our net losses may fluctuate significantly from quarter to quarter and from year to year. We anticipate that our expenses will increase significantly as we conduct clinical trials for MRX34 and other product candidates; manufacture clinical trial materials; continue to discover, validate and develop additional novel product candidates; expand and protect our intellectual property portfolio; and hire additional development and scientific personnel.

Financial Operations Overview

Revenue

We have not generated any revenue from product sales or from collaborations. In the future, we may generate revenue from collaborations and licenses. Revenue may fluctuate from period to period, and the timing and extent of any future revenue will depend on our ability to advance our product candidates through the clinical trial process and to obtain regulatory approval and our ability, or our future partners' ability, to commercialize our product candidates.

Research and Development Expenses

Research and development expenses consist primarily of costs incurred for our research activities, including our drug discovery efforts, and the development of our product candidates, which include the following:

- employee-related expenses, including salaries, benefits, travel and stock-based compensation;
- external research and development expenses incurred under arrangements with third parties, such as contract research organizations, or CROs, consultants and our scientific advisory board;
- lab supplies, and acquiring, developing and manufacturing preclinical study materials in accordance with Good Laboratory Practices;
- costs of clinical trials, including costs for management, investigator fees and related vendors that provide services for the clinical trials;
- costs to manufacture the drug used in the clinical trials in accordance with Good Manufacturing Practices;
- license and milestone fees;
- development and prosecution of intellectual property; and
- costs of facilities, depreciation and other expenses.

Research and development costs are expensed as incurred. In certain circumstances, we will make nonrefundable advance payments to purchase goods and services for future use pursuant to contractual arrangements. In those instances, we defer and recognize an expense in the period that we receive or consume the goods or services.

Our research and development expenses have been offset by proceeds derived from federal and state grants. These government grants, which have supplemented our research efforts on specific projects, generally provide for reimbursement of approved costs, as defined in the terms of the grant awards. The proceeds from these reimbursement grants are treated as a reduction to the associated expenses as the allowable expenses are incurred.

At any point in time, we typically have various early stage research and drug discovery projects ongoing. Our internal resources, employees and infrastructure are not directly tied to any one research or drug discovery project and are typically deployed across multiple projects. As such, we do not maintain information regarding the costs incurred for these early stage research and drug discovery programs on a project-specific basis. However, we have spent and are currently spending the vast majority of our research and development resources on our lead product candidate, MRX34.

Most of our product development programs are at an early stage, and successful development of future product candidates from these programs is highly uncertain and may not result in approved products. The process of conducting preclinical studies and clinical trials necessary to obtain regulatory approval is costly and time consuming, and we expect our research and development expenses to increase for the foreseeable future as we advance our research programs toward the clinic and initiate and continue clinical trials. The probability of success for each product candidate may be affected by numerous factors, including preclinical data, clinical data, competition, manufacturing capability and commercial viability. We anticipate we will make determinations as to which programs to pursue and how much funding to direct to each program on an ongoing basis in response to the scientific and clinical success of each future product candidate, as well as ongoing assessments as to each future product candidate's commercial potential. Completion dates and completion costs can vary significantly for each future product candidate and are difficult to predict. We will need to raise additional capital and may seek strategic alliances in the future in order to advance the various products in the pipeline and other products that may be developed.

General and Administrative Expenses

General and administrative expenses consist primarily of salaries and related benefits, including stock-based compensation, related to our executive, finance and support functions. Other general and administrative expenses include allocated facility-related costs not otherwise included in research and development expenses, travel expenses and professional fees for auditing, tax and legal services. We expect that general and administrative expenses will increase in the future as we expand our operating activities and incur additional costs associated with being a publicly-traded company. These increases will likely include legal fees, accounting fees, directors' and officers' liability insurance premiums and fees associated with investor relations.

Critical Accounting Policies and Estimates

Our management's discussion and analysis of our financial condition and results of operations are based on our financial statements, which have been prepared in accordance with generally accepted accounting principles. The preparation of these financial statements requires us to make estimates and judgments that affect the reported amounts of assets, liabilities, and expenses and the disclosure of contingent assets and liabilities in our financial statements. On an ongoing basis, we evaluate our estimates and judgments, including those related to stock based compensation and clinical trial and pre-clinical study accruals. We base our estimates on historical experience, known trends and events and various other factors that are believed to be reasonable under the circumstances, the results of which form the basis for making judgments about the carrying values of assets and liabilities that are not readily apparent from other sources. Actual results may differ from these estimates under different assumptions or conditions. In making estimates and judgments, management employs critical accounting policies. During the three months ended March 31, 2016, there were no material changes to our critical accounting policies as reported in our Annual Report on Form 10-K for the year ended December 31, 2015, which was filed with the SEC on March 30, 2016.

Results of Operations

Comparison of three months ended March 31, 2016 and 2015:

	Three Months Ended March 31,		Dollar Change	% Change
	2016	2015		
	(in thousands)			
Statement of operations data:				
Operating expenses:				
Research and development, before grant reimbursement	$ 4,563	$ 3,475	$ 1,088	31.3 %
Less grant reimbursement	(40)	(73)	33	(45.2)%
Research and development	4,523	3,402	1,121	33.0 %
General and administrative	2,130	877	1,253	142.9 %
Interest (income)	(82)	—	(82)	100.0 %
Net loss	$ 6,571	$ 4,279	$ 2,292	53.6 %

Research and Development Expenses

Research and development expenses were $4.5 million for the three months ended March 31, 2016 which was an increase of $1.1 million, or 33%, compared to research and development expenses of approximately $3.4 million for the three months ended March 31, 2015.

Research and development spending, prior to the offset of grant reimbursements, was $4.6 million for the three months ended March 31, 2016, which was an increase of approximately $1.1 million, or 31%, compared to research and development spending, prior to the offset of grant reimbursements, of $3.5 million for the three months ended March 31, 2016. The increase in the three months ended March 31, 2016 was primarily due to the following:

- Approximately $733,000 of increased employee compensation and benefits expense, of which $594,000 related to increased payroll and benefits expense and $139,000 related to stock-based compensation expense.
- Approximately $285,000 of increased clinical trial costs related to our Phase 1 clinical trial, including administrative and testing costs, additional investigator sites and additional drug costs related to the advanced trial activity, as well as increased intellectual property and licensing costs.

Research and development spending was partially offset by approximately $40,000 of grant reimbursements for the three months ended March 31, 2016, compared to reimbursement of approximately $73,000 for the same period in 2015. The decrease is primarily due to two grants expiring in August 2015.

General and Administrative Expenses

General and administrative expenses were approximately $2.1 million for the three months ended March 31, 2016, which was an increase of approximately $1.3 million or 143%, compared to general and administrative expenses of $877,000 for the three months ended March 31, 2015. The increase in the three months ended March 31, 2016 was primarily due to the following:

- Approximately $518,000 for increased employee compensation and benefits expense, of which $345,000 related to increased payroll and benefits expense, and $173,000 related to stock based compensation expense.

- Approximately $466,000 for additional costs associated with operating as a publicly traded company, including higher legal, audit, insurance and administrative costs.

Liquidity and Capital Resources

Liquidity and Capital Expenditures

Since inception, our operations have been financed primarily through proceeds of $167.3 million to fund our operations, of which approximately $89.9 million was from the issuance of preferred stock for cash and assets, $48.7 million from a public offering of our common stock, $16.8 million from a private placement of our common stock and $11.9 million was from federal and state grants. At March 31, 2016, we had $52.9 million of cash and cash equivalents. Our primary uses of cash are to fund operating expenses, primarily research and development expenditures. Cash used to fund operating expenses is impacted by the timing of when we pay these expenses, as reflected in the change in our outstanding accounts payable and accrued expenses.

We believe that our existing cash and cash equivalents as of March 31, 2016, will be sufficient to meet our anticipated cash requirements for at least the next 12 months. However, our forecast of the period of time through which our financial resources will be adequate to support our operations is a forward-looking statement that involves risks and uncertainties, and actual results could vary materially.

Our future capital requirements are difficult to forecast and will depend on many factors, including:

· the initiation, progress, timing and completion of preclinical studies and clinical trials for our lead product and potential product candidates;

· the number and characteristics of product candidates that we pursue;

· the progress, costs and results of our clinical trials;

· the terms and timing of any other strategic alliance, licensing and other arrangements that we may establish;

· the outcome, timing and cost of regulatory approvals;

· delays that may be caused by changing regulatory requirements;

· the costs and timing of any possible future defense of our intellectual property or decisions to acquire additional intellectual property;

· the costs of developing and maintaining our intellectual property portfolio;

· the costs and timing of hiring new employees to support our continued growth;

· the costs and timing of procuring clinical supplies of our product candidates; and

· the extent to which we acquire or invest in businesses, products or technologies.

The following table shows a summary of our cash flows for the three months ended March 31, 2016 and 2015.

	Three Months Ended March 31,	
	2016	2015
	(in thousands)	
Net cash provided by (used in):		
Operating activities	$ (8,973)	$ (4,550)
Investing activities	(27,865)	(5)
Financing activities	—	35,366
Net increase (decrease)	$ (36,838)	$ 30,811

Operating Activities

Net cash used in operating activities was $9.0 million and $4.6 million for the three months ended March 31, 2016 and 2015, respectively. The increase in overall spending for operating activities of approximately $4.4 million was due to increased headcount and personnel expenses, increased spending for clinical trials and intellectual property related expenses.

Investing Activities

Net cash used in investing activities for the periods presented relates primarily to the purchase of marketable securities during the three months ended March 31, 2016. We invested $27.7 million in US government agency and treasury securities and corporate debt securities with maturities of less than 180 days using surplus proceeds received in connection with the IPO and concurrent private placement in October 2015. For the three months ended March 31, 2016 and 2015 total amounts spent on the purchase of fixed assets were approximately $143,000 and $5,000, respectively

Financing Activities

Net cash provided by financing activities was approximately $35.4 million for the three months ended March 31, 2015, which was attributable to the initial closing of our offering of Series D convertible preferred stock. There were no financing activities during the three months ended March 31, 2016.

Contractual Obligations and Commitments

During the three months ended March 31, 2016, there were no material changes to our contractual obligations and commitments described under Management's Discussion and Analysis of Financial Condition and Results of Operations included in our Annual Report on Form 10-K for the year ended December 31, 2015, which was filed with the Securities and Exchange Commission on March 30, 2016.

Off-balance Sheet Arrangements

We did not have during the periods presented, and we do not currently have, any off-balance sheet arrangements, as defined in the rules and regulations of the Securities and Exchange Commission.

Segment Information

We have one primary business activity and operate as one reportable segment.

JOBS Act

Section 107 of the JOBS Act provides that an emerging growth company can take advantage of the extended transition period provided in Section 7(a)(2)(B) of the Securities Act for complying with new or revised accounting standards. Thus, an emerging growth company can delay the adoption of certain accounting standards until those standards would otherwise apply to private companies. We have irrevocably elected not to avail ourselves of this

extended transition period and, as a result, we will adopt new or revised accounting standards on the relevant dates on which adoption of such standards is required for other companies.

Item 3. Quantitative and Qualitative Disclosures about Market Risk

The market risk inherent in our financial instruments and in our financial position represents the potential loss arising from adverse changes in interest rates. At March 31, 2016, we had cash and cash equivalents and marketable securities of $52.9 million and $27.7 million, respectively, consisting of interest-bearing money market funds, U.S. treasury securities, U.S. government agency securities, and corporate debt securities. Our primary exposure to market risk is interest rate sensitivity, which is affected by changes in the general level of U.S. interest rates. Due to the short-term maturities of our cash equivalents and marketable securities, as well as the low risk profile of our investments, we do not believe a change in interest rates would have a material effect on the fair market value of our cash and cash equivalents and marketable securities.

Item 4. Controls and Procedures.

Evaluation of Disclosure Controls and Procedures

Our management, with the participation of our chief executive and financial officers, evaluated the effectiveness of our disclosures controls and procedures, as defined in Rules 13a-15(e) and 15d-15(e) under the Exchange Act, as of March 31, 2016. The term “disclosure controls and procedures,” as defined in Rules 13a-15(e) and 15d-15(e) under the Exchange Act, means controls and other procedures of a company that are designed to ensure that information required to be disclosed by a company in the reports that it files or submits under the Exchange Act is recorded, processed, summarized and reported within the time periods specified in the SEC’s rules and forms. Disclosure controls and procedures include, without limitation, controls and procedures designed to ensure that information required to be disclosed by a company in the reports that it files or submits under the Exchange Act is accumulated and communicated to the company’s management, including its principal executive and principal financial officers, as appropriate, to allow timely decisions regarding required disclosure. Management recognizes that any controls and procedures, no matter how well designed and operated, can provide only reasonable assurance of achieving their objectives and management necessarily applies its judgment in evaluating the cost-benefit relationship of possible controls and procedures. Based on the evaluation of our disclosure controls and procedures as of March 31, 2016, our chief executive officer and chief financial officer concluded that, as of such date, our disclosure controls and procedures were effective at a reasonable assurance level.

Changes in Internal Control over Financial Reporting

There were no changes in our internal controls over financial reporting during the period covered by this Quarterly Report on Form 10-Q identified in connection with the evaluation required by Rule 13a-15(d) and 15d-15(d) of the Exchange Act that has materially affected, or is reasonably likely to materially affect, our internal control over financial reporting.

PART II: OTHER INFORMATION

Item 1. Legal Proceedings

We are not currently a party to any material legal proceedings. We may at times be involved in litigation and other legal claims in the ordinary course of business. When appropriate in management's estimation, we may record reserves in our financial statements for pending litigation and other claims.

Item 1A. Risk Factors

Investing in our common stock involves a high degree of risk. You should consider carefully the following risks, together with all the other information in this periodic report, including our financial statements and notes thereto and "Management's Discussion and Analysis of Financial Condition and Results of Operations," before you invest in our common stock. If any of the following risks actually materializes, our operating results, financial condition and liquidity could be materially adversely affected. As a result, the trading price of our common stock could decline and you could lose part or all of your investment. Additional risks and uncertainties not presently known to us or that we currently deem immaterial also may impair our business operations.

Risk Factors

Risks Related to Our Limited Operating History, Financial Position and Capital Requirements

We have incurred significant losses since inception. We anticipate that we will continue to incur significant losses for the foreseeable future, and if we are unable to achieve and sustain profitability, the market value of our common stock will likely decline.

We are a clinical-stage biopharmaceutical company with a limited operating history. Biopharmaceutical product development is a highly speculative undertaking and involves a substantial degree of risk. We have not generated any product revenues and we do not expect to generate any product revenues for the foreseeable future. We have incurred losses in each year since our founding in 2007 and we expect to continue to incur significant operating losses for the foreseeable future. The amount of future losses is uncertain. All of our product candidates are in development, and none has been approved for sale. We have devoted substantially all of our efforts to research and development, including our preclinical and nonclinical development activities, and expect that it will be many years, if ever, before we have a product candidate ready for commercialization. To date, we have derived all of our funding from our collaboration with our former parent company, Asuragen, Inc., or Asuragen, private placements of preferred stock and government grants for research and development. Our net loss for the three months ended March 31, 2016 was $6.6 million. Since inception, we have incurred net losses leading to an accumulated deficit of approximately $83.1 million as of March 31, 2016.

We expect to continue to incur significant expenses and increasing operating losses for the foreseeable future as we expand our clinical development plan for MRX34 as a monotherapy, pursue development of MRX34 as a combination therapy, conduct research and development of other product candidates and pursue marketing approval for MRX34 in the future. If we obtain marketing approval of MRX34, we also expect to incur significant sales, marketing, distribution and manufacturing expenses. Even after obtaining such marketing approval, our products may never gain sufficient market acceptance and adequate market share. If we fail to succeed in any of these activities or our product candidates fail to demonstrate safety and efficacy in clinical trials, do not gain regulatory approval or do not achieve significant market acceptance following regulatory approval and commercialization, we may never become profitable. Even if we achieve profitability in the future, we may not be able to sustain profitability in subsequent periods. Our prior losses, combined with expected future losses, have had and will continue to have an adverse effect on our stockholders' equity and working capital. If we are unable to achieve and sustain profitability, the market value of our common stock will likely decline. Because of the numerous risks and uncertainties associated with developing biopharmaceutical products, we are unable to predict the extent of any future losses or whether we will become profitable.

Our short operating history may make it difficult for you to evaluate the success of our business to date and to assess our future viability.

We are a clinical-stage biopharmaceutical company that was founded in 2007 and did not exist as a standalone company until 2009. Our operations to date have been limited to organizing and staffing our company, business planning, raising capital, acquiring and developing our technology, identifying and evaluating potential product candidates and delivery technologies, undertaking nonclinical studies, filing an Investigational New Drug, or IND, application with the U.S. Food and Drug Administration, or FDA, and conducting the Phase 1 clinical trial of our most advanced product candidate, MRX34. Except for MRX34, all of our product candidates are still in preclinical development. We have not yet demonstrated our ability to initiate clinical trials for product candidates other than MRX34, or successfully complete any clinical trials, including large-scale, pivotal clinical trials, obtain marketing approvals, manufacture a commercial scale medicine, or arrange for a third party to do so on our behalf, or conduct sales and marketing activities necessary for successful commercialization. Typically, it takes many years to develop one new product candidate from the time it is discovered to when it is available for treating patients. Consequently, any predictions about our future success or viability, or any evaluation of our business or prospects, may not be as accurate as they could be if we had a longer operating history. In addition, as a new business, we may encounter unforeseen expenses, difficulties, complications, delays and other known and unknown challenges. We will need to transition from a company with a research focus to a company capable of supporting commercial activities. We may not be successful in such a transition.

We will need substantial additional funding. If we are unable to raise capital when needed, we could be forced to delay, reduce or terminate our product development, other operations or commercialization efforts.

Developing biopharmaceutical products, including conducting preclinical and nonclinical studies and clinical trials, is an expensive and highly uncertain process that takes years to complete. Our expenses will increase substantially as we expand our clinical development plan for MRX34 as a monotherapy, pursue development of MRX34 as a combination therapy, conduct research and development of other product candidates and pursue marketing approval for MRX34 in the future. Additional clinical trials, including one or more late-stage pivotal trials, will be required to obtain potential marketing approval for MRX34, and the costs of any future trials may be more expensive and time consuming than our current trial. If we obtain marketing approval of MRX34, we also expect to incur significant sales, marketing, distribution and outsourced manufacturing expenses.

As of March 31, 2016, we had working capital of $78.2 million and cash and cash equivalents of $52.9 million. Based on our current operating plan, we believe that our available cash at such date are sufficient to fund our anticipated levels of operation for at least the next 12 months. Our future capital requirements for the period for which we expect our existing resources to support our operations may vary significantly from what we expect. For example, our expenses could increase beyond expectations if we are required by the FDA or comparable foreign regulatory agencies to perform studies and trials in addition to those that we currently anticipate. Our funds at March 31, 2016 will not be sufficient to obtain marketing approval for MRX34. As a result, we will be required to obtain additional financing in the future, which we may obtain through public or private equity offerings, debt financings, a credit facility, government grants and contracts and/or strategic collaborations. If we are required to secure additional capital, such additional fundraising efforts may divert our management from our day-to-day activities, which may adversely affect our ability to develop and commercialize future product candidates. Additional financing may not be available to us when we need it or it may not be available to us on favorable terms, if at all. If we are unable to obtain adequate financing or form favorable collaborations, when needed, we may have to delay, reduce the scope of or eliminate one or more of our clinical trials, research and development programs or our commercialization efforts, including with respect to MRX34.

Additionally, our future financing requirements will depend on many factors, some of which are beyond our control, including:

- the demonstration of further clinical proof-of-concept with our product candidates, including MRX34, in one or more cancer types or other indications;

- the rate of progress and cost of our clinical trials, preclinical and nonclinical studies and other discovery and research and development activities;
- the successful outcome of one or more pivotal clinical trials demonstrating safety and efficacy of our product candidates, including MRX34;
- the timing of, and costs involved in, seeking and obtaining FDA and other regulatory approvals;
- the costs of preparing, filing, prosecuting, maintaining and enforcing any patent claims and other intellectual property rights, including litigation costs and the results of such litigation;
- our ability to practice our technology without infringing the intellectual property rights of third parties;
- our ability to enter into additional collaboration, licensing, government or other arrangements and the terms and timing of such arrangements;
- the potential need to acquire, by acquisition or in-licensing, other products or technologies; and
- the emergence of competing technologies or other adverse market developments.

Future capital requirements will also depend on the extent to which we acquire or invest in additional complementary businesses, products and technologies. We currently have no understandings, commitments or agreements relating to any of these types of transactions.

Raising additional capital may cause dilution to our existing stockholders, restrict our operations or require us to relinquish rights to our technologies or product candidates.

Until such time, if ever, as we can generate substantial product revenues, we expect to finance our cash needs through public or private equity offerings, debt financings, credit facilities, government grants and contracts and/or strategic collaborations.

To raise capital, we may from time to time issue additional shares of common stock at a discount from the then-current trading price of our common stock. As a result, our common stockholders would experience immediate dilution upon the purchase of any shares of our common stock sold at such discount. In addition, as opportunities present themselves, we may enter into financing or similar arrangements in the future, including the issuance of debt securities, preferred stock or common stock. Whether or not we issue additional shares of common stock at a discount, any issuance of common stock will, and any issuance of other equity securities, securities convertible into equity securities or options, warrants or other rights to purchase common stock may, result in additional dilution of the percentage ownership of our stockholders and could cause our stock price to decline. New investors could also gain rights, preferences and privileges senior to those of holders of our common stock, which could cause the price of our common stock to decline. Debt securities may also contain covenants that restrict our operational flexibility, impose liens or other restrictions on our assets, restrict our ability to incur additional debt, impose limitations on our ability to acquire, sell or license intellectual property or impose other operating restrictions that could adversely affect our business and could also cause the price of our common stock to decline.

Other than our collaboration with our former parent company, Asuragen, private placements of preferred stock, and offerings of common stock, the only external source of funds to date has been state and federal government grants for research and development. The grants have been, and any future government grants and contracts we may receive may be, subject to the risks and contingencies set forth below under the risk factor entitled “Reliance on government funding for our programs may add uncertainty to our research and commercialization efforts with respect to those programs that are tied to such funding and may impose requirements that limit our ability to take certain actions, increase the costs of commercialization and production of product candidates developed under those programs and subject us to potential financial penalties, which could materially and adversely affect our business, financial condition and results of operations.” Although we might apply for government and private contracts and grants in the future, we cannot assure

you that we will be successful in obtaining additional grants or contracts for MRX34 or any other product candidates or programs.

Risks Related to Product Development and Commercialization

The approach we are taking to discover and develop novel therapeutics using microRNA is unproven and may never lead to marketable products.

The scientific discoveries that form the basis for our efforts to discover and develop new drugs are relatively recent. To date, neither we nor any other company has received regulatory approval to market therapeutics utilizing microRNA. The scientific evidence to support the feasibility of developing drugs based on these discoveries is both preliminary and limited. Successful development of microRNA-based products by us will require solving a number of issues, including providing suitable methods of stabilizing the microRNA material and delivering it into target cells in the human body. In addition, any compounds that we develop may not demonstrate in patients the chemical and pharmacological properties ascribed to them in laboratory and nonclinical studies, and they may interact with human biological systems in unforeseen, ineffective or even harmful ways. If we do not successfully develop and commercialize product candidates based upon our technological approach, we may not become profitable and the value of our common stock may decline.

Further, the FDA has relatively limited experience with microRNA-based therapeutics. No regulatory authority has granted approval to any person or entity, including us, to market and commercialize microRNA therapeutics, which may increase the complexity, uncertainty and length of the regulatory approval process for our product candidates. If our microRNA technologies prove to be ineffective, unsafe or commercially unviable, our entire pipeline would have little, if any, value, which would have a material adverse effect on our business, financial condition, results of operations and prospects.

Further, our exclusive focus on microRNA technology for developing products as opposed to multiple, more proven technologies for drug development increases the risk associated with our business. If we are not successful in developing a product candidate using microRNA technology, we may not be able to identify and successfully implement an alternative product development strategy.

We are heavily dependent on the success of our lead product candidate, MRX34, which is in Phase 1 clinical development.

We currently have no products approved for sale and have invested a significant portion of our efforts and financial resources in the development of MRX34. The clinical development of MRX34 began in April 2013 with a multi-center Phase 1 clinical trial that is currently enrolling patients with advanced stage solid cancers. We have also included in the Phase 1 clinical trial a separate cohort of patients with hematological malignancies, which may include patients with non-Hodgkin’s lymphoma, acute myelogenous leukemia, acute and chronic lymphocytic leukemia, chronic myelogenous leukemia in accelerated or blast phase, multiple myeloma and myelodysplastic syndrome. The primary objectives of the Phase 1 clinical trial, including the hematological malignancy cohort, are to establish the maximum tolerated dose and an appropriate dose for Phase 2 clinical trials. The secondary objectives of the Phase 1 clinical trial are to assess the safety, tolerability and pharmacokinetic profile of MRX34 after intravenous dosing as well as to assess any biological and clinical activity.

Our prospects are substantially dependent on our ability to develop and commercialize MRX34. Our ability to timely develop and effectively commercialize MRX34 will depend on several factors, including the following:

- successful completion of our Phase 1 clinical trial or other clinical trials, which will depend substantially upon the satisfactory performance of third-party contractors;

- successful demonstration of further clinical proof-of-concept with MRX34 in one or more cancer types;

- successful outcome of one or more pivotal clinical trials required for regulatory approval demonstrating safety and efficacy of MRX34;
- receipt of marketing approvals for MRX34 from the FDA and similar regulatory authorities outside the United States;
- establishing commercial manufacturing capabilities, for example, by making arrangements with third-party manufacturers;
- successfully launching commercial sales of the product, whether alone or in collaboration with others;
- acceptance of the product by patients, the medical community and third-party payors;
- establishing market share while competing with other therapies;
- a continued acceptable safety and adverse event profile of the product following regulatory approval;
- qualifying for, identifying, registering, maintaining, enforcing and defending intellectual property rights and claims covering the product; and
- manufacturing, marketing, selling and using MRX34 and practicing our technology without infringing the proprietary rights of third parties, or successfully defending against claims alleging such infringement.

If we do not achieve one or more of these factors in a timely manner, or at all, we could experience significant delays or an inability to commercialize MRX34, which would materially and adversely affect our business, financial condition and results of operations.

We have not previously submitted a new drug application, or NDA, to the FDA, or similar drug approval filings to comparable foreign authorities, for any product candidate, and we cannot be certain that any of our product candidates will be successful in clinical trials or receive regulatory approval. Further, our product candidates may not receive regulatory approval even if they are successful in clinical trials. If we do not receive regulatory approvals for our product candidates, we may not be able to continue our operations. Even if we successfully obtain regulatory approvals to market one or more of our product candidates, our revenues will be dependent upon the size of the markets in the territories for which we gain regulatory approval and have commercial rights. If the markets for patient subsets that we are targeting are not as significant as we estimate, we may not generate significant revenues from sales of such products, if approved. Successful development of MRX34 or other product candidates for additional indications will be subject to these same risks.

If we are not successful in discovering, developing and commercializing additional product candidates, our ability to expand our business and achieve our strategic objectives would be impaired.

Although a substantial amount of our efforts will focus on the MRX34 Phase 1 clinical trial and the initiation of several Phase 2 studies, a key element of our strategy is to discover, develop and potentially commercialize a portfolio of product candidates to treat cancer and other indications. We are seeking to do so through our internal research programs and are exploring, and intend to explore in the future, strategic partnerships for the development of new products. Other than MRX34, all of our other potential product candidates remain in the discovery and preclinical study stages. Research programs to identify product candidates require substantial technical, financial and human resources, whether or not any product candidates are ultimately identified. Our research programs may initially show promise in identifying potential product candidates, yet fail to yield product candidates for clinical development for many reasons, including the following:

- the research methodology used may not be successful in identifying potential product candidates;
- competitors may develop alternatives that render our product candidates obsolete;

- product candidates we develop may nevertheless be covered by third parties' patents or other exclusive rights;
- a product candidate may, on further study, be shown to have harmful side effects or other characteristics that indicate it is unlikely to be effective or otherwise does not meet applicable regulatory criteria;
- a product candidate may not be capable of being produced in commercial quantities at an acceptable cost, or at all; and
- a product candidate may not be accepted as safe and effective by patients, the medical community or third-party payors.

If we are unsuccessful in identifying and developing additional product candidates, our potential for growth may be impaired.

We may use our financial and human resources to pursue a particular research program or product candidate and fail to capitalize on programs or product candidates that may be more profitable or for which there is a greater likelihood of success.

Because we have limited financial and human resources, we may forego or delay pursuit of opportunities with certain programs or product candidates or for other indications that later prove to have greater commercial potential. Our resource allocation decisions may cause us to fail to capitalize on viable commercial products or more profitable market opportunities. Our spending on current and future research and development programs and future product candidates for specific indications may not yield any commercially viable products. We may also enter into strategic alliance agreements to develop and commercialize certain of our programs and potential product candidates in indications with potentially large commercial markets. If we do not accurately evaluate the commercial potential or target market for a particular product candidate, we may relinquish valuable rights to that product candidate through strategic alliance, licensing or other royalty arrangements in cases in which it would have been more advantageous for us to retain sole development and commercialization rights to such product candidate, or we may allocate internal resources to a product candidate in a therapeutic area in which it would have been more advantageous to enter into a partnering arrangement.

The regulatory approval process is lengthy, expensive and uncertain, and we may be unable to obtain regulatory approval for our drug products under applicable regulatory requirements. The denial or delay of any such approval would delay commercialization of our drug products and adversely impact our ability to generate revenue, our business and our results of operations.

The development, research, testing, manufacturing, labeling, approval, selling, import, export, marketing and distribution of drug products are subject to extensive and evolving regulation by federal, state and local governmental authorities in the United States, principally the FDA, and by foreign regulatory authorities, which regulations differ from country to country. Neither we nor any future collaborator is permitted to market MRX34 or any other product candidate in the United States until we receive regulatory approval of an NDA from the FDA.

Obtaining regulatory approval of an NDA can be a lengthy, expensive and uncertain process. Prior to obtaining approval to commercialize a drug candidate in the United States or abroad, we or our collaborators must demonstrate with substantial evidence from well-controlled clinical trials, and to the satisfaction of the FDA or other foreign regulatory agencies, that such drug candidates are safe and effective for their intended uses. The number of nonclinical studies and clinical trials that will be required for FDA approval varies depending on the drug candidate, the disease or condition that the drug candidate is designed to address, and the regulations applicable to any particular drug candidate. Results from nonclinical studies and clinical trials can be interpreted in different ways. Even if we believe the nonclinical or clinical data for our drug candidates are promising, such data may not be sufficient to support approval by the FDA and other regulatory authorities. Administering drug candidates to humans may produce undesirable side effects, which could interrupt, delay or halt clinical trials and result in the FDA or other regulatory authorities denying approval of a drug candidate for any or all indications. The FDA may also require us to conduct additional studies or trials for our

product candidates either prior to or post-approval, such as additional drug-drug interaction studies or safety or efficacy studies or trials, or it may object to elements of our clinical development program such as the number of subjects in our current clinical trials from the United States.

We expect to complete enrollment in the Phase 1 clinical trial and to initiate enrollment in a Phase 1b translational medicine study and the Phase 2 clinical trials for our lead product candidate, MRX34, by the end of 2016, and our business currently depends substantially on the successful development, regulatory approval and commercialization of MRX34. We currently have no drug products approved for sale, and we may never obtain regulatory approval to commercialize MRX34.

The FDA or any foreign regulatory bodies can delay, limit or deny approval of MRX34 or require us to conduct additional nonclinical or clinical testing or abandon a program for many reasons, including:

- the FDA or the applicable foreign regulatory agency's disagreement with the design or implementation of our clinical trials;
- negative or ambiguous results from our clinical trials or results that may not meet the level of statistical significance required by the FDA or comparable foreign regulatory agencies for approval;
- serious and unexpected drug-related side effects experienced by participants in our clinical trials or by individuals using drugs similar to our product candidates;
- our inability to demonstrate to the satisfaction of the FDA or the applicable foreign regulatory body that MRX34 is safe and effective for the proposed indication;
- the FDA's or the applicable foreign regulatory agency's disagreement with the interpretation of data from nonclinical studies or clinical trials;
- our inability to demonstrate the clinical and other benefits of MRX34 outweigh any safety or other perceived risks;
- the FDA's or the applicable foreign regulatory agency's requirement for additional nonclinical studies or clinical trials;
- the FDA's or the applicable foreign regulatory agency's disagreement regarding the formulation, labeling and/or the specifications of MRX34;
- the FDA's or the applicable foreign regulatory agency's failure to approve the manufacturing processes or facilities of third-party manufacturers with which we contract; or
- the potential for approval policies or regulations of the FDA or the applicable foreign regulatory agencies to significantly change in a manner rendering our clinical data insufficient for approval.

Of the large number of drugs in development, only a small percentage successfully complete the FDA or other regulatory approval processes and are commercialized. The lengthy approval process as well as the unpredictability of future clinical trial results may result in our failing to obtain regulatory approval to market MRX34, which would significantly harm our business, financial condition, results of operations and prospects.

Even if we eventually complete clinical testing and receive approval of an NDA or foreign marketing application for MRX34, the FDA or the applicable foreign regulatory agency may grant approval contingent on the performance of costly additional clinical trials, including Phase 4 clinical trials, and/or the implementation of a Risk Evaluation and Mitigation Strategy, or REMS, which may be required to ensure safe use of the drug after approval. The FDA or the applicable foreign regulatory agency also may approve MRX34 for a more limited indication or a narrower patient population than we originally requested, and the FDA or applicable foreign regulatory agency may not approve

the labeling that we believe is necessary or desirable for the successful commercialization of MRX34. Any delay in obtaining, or inability to obtain, applicable regulatory approval would delay or prevent commercialization of MRX34 and would materially adversely impact our business and prospects.

Clinical drug development involves a lengthy and expensive process with an uncertain outcome, and results of earlier studies and trials may not be predictive of future trial results.

Clinical testing is expensive and can take many years to complete, and its outcome is inherently uncertain. Furthermore, we rely on contract research organizations, or CROs, and clinical trial sites to ensure the proper and timely conduct of our clinical trials. While we have agreements with our CROs governing their committed activities, and the ability to audit their performance, we have limited influence over their actual performance. Failure or delay can occur at any time during the clinical trial process. Success in nonclinical testing and early clinical trials does not ensure that later clinical trials will be successful, and the results of clinical trials by other parties may not be indicative of the results in trials we may conduct. A number of companies in the pharmaceutical industry, including biotechnology companies, have suffered significant setbacks in clinical trials, even after promising results in earlier nonclinical or clinical studies. These setbacks have been caused by, among other things, nonclinical findings made while clinical studies were underway and safety or efficacy observations made in clinical studies, including previously unreported adverse events. The results of preclinical, nonclinical and early clinical studies of our product candidates may not be predictive of the results of later-stage clinical trials. Product candidates in later stages of clinical trials may fail to show the desired safety and efficacy traits despite having progressed through preclinical and initial clinical trials. Notwithstanding any potential promising results in earlier studies, we cannot be certain that we will not face similar setbacks. Even if our clinical trials are completed, the results may not be sufficient to obtain regulatory approval for our product candidates.

Although we have an ongoing Phase 1 clinical trial for MRX34 that is expected to complete enrollment and planned Phase 1b translational medicine study and Phase 2 clinical trials that are expected to initiate enrollment by the end of 2016, we may experience delays in these trials and we cannot be certain that the trial or any other future clinical trials for MRX34 or other product candidates will begin on time, need to be redesigned, enroll an adequate number of patients on time or be completed on schedule, if at all. Clinical trials can be delayed or aborted for a variety of reasons, including delay or failure related to:

- the FDA or comparable foreign regulatory authorities disagreeing as to the design or implementation of our clinical studies;
- obtaining regulatory approval to commence a trial;
- reaching agreement on acceptable terms with prospective CROs and clinical trial sites, the terms of which can be subject to extensive negotiation and may vary significantly among different CROs and trial sites;
- obtaining institutional review board, or IRB, or equivalent approval at each site;
- recruiting suitable patients to participate in a trial;
- having patients complete a trial or return for post-treatment follow-up;
- clinical sites deviating from trial protocol or dropping out of a trial;
- addressing patient safety concerns that arise during the course of a trial;
- addressing any conflicts with new or existing laws or regulations;
- adding a sufficient number of clinical trial sites; or
- manufacturing sufficient quantities of product candidate for use in clinical trials.

Patient enrollment is a significant factor in the timing of clinical trials and is affected by many factors, including the size and nature of the patient population, the proximity of patients to clinical sites, the eligibility criteria for the trial, the design of the clinical trial, competing clinical trials and clinicians' and patients' perceptions as to the potential advantages of the drug being studied in relation to other available therapies, including any new drugs or treatments that may be approved for the indications we are investigating.

We could also encounter delays if a clinical trial is suspended or terminated by us, by the IRBs of the institutions in which such trials are being conducted, by the Data Safety Monitoring Board, or DSMB, for such trial or by the FDA or other regulatory authorities. Such authorities may suspend or terminate a clinical trial due to a number of factors, including failure to conduct the clinical trial in accordance with regulatory requirements or our clinical protocols, inspection of the clinical trial operations or trial site by the FDA or other regulatory authorities resulting in the imposition of a clinical hold, unforeseen safety issues or adverse side effects, failure to demonstrate a benefit from using a drug, changes in governmental regulations or administrative actions or lack of adequate funding to continue the clinical trial.

Further, conducting clinical trials in foreign countries, as we currently do for MRX34, presents additional risks that may delay completion of our clinical trials. These risks include the failure of enrolled patients in foreign countries to adhere to clinical protocol as a result of differences in healthcare services or cultural customs, managing additional administrative burdens associated with foreign regulatory schemes, as well as political and economic risks relevant to such foreign countries.

If we experience delays in the completion, or termination, of any clinical trial of our product candidates, the commercial prospects of our product candidates may be harmed, and our ability to generate product revenues from any of these product candidates will be delayed or not realized at all. In addition, any delays in completing our clinical trials will increase our costs, slow down our product candidate development and approval process and jeopardize our ability to commence product sales and generate revenues. Any of these occurrences may significantly harm our business, financial condition and prospects. In addition, many of the factors that cause, or lead to, a delay in the commencement or completion of clinical trials may also ultimately lead to the denial of regulatory approval of our product candidates.

If we are required to suspend or discontinue clinical trials due to side effects or other safety risks, or if we are required to conduct studies on the long-term effects associated with the use of MRX34 or other product candidates, our ability to continue developing or commercialize our product candidates could be adversely affected.

Our clinical trials, including our Phase 1 clinical trial for MRX34, the planned initiation of a Phase 1b translational medicine study and several Phase 2 studies, or other trials our strategic partners or CROs may conduct, may be suspended or terminated at any time for a number of safety-related reasons. For example, we may voluntarily suspend or terminate our clinical trials if at any time we believe that our product candidates present an unacceptable safety risk to the clinical trial patients. In addition, IRBs or regulatory agencies may order the temporary discontinuation or termination of our clinical trials at any time if they believe that the clinical trials are not being conducted in accordance with applicable regulatory requirements, including if they present an unacceptable safety risk to patients. Administering any product candidate to humans may produce undesirable side effects. The existence of undesirable side effects resulting from our product candidates could cause us or regulatory authorities, such as the FDA, to interrupt, delay or halt clinical trials of our product candidates and could result in the FDA or other regulatory agencies denying further development or approval of our product candidates for any or all indications.

We have not conducted complete studies on the long-term effects associated with the use of MRX34 or any other product candidate. Studies of these long-term effects may be required for regulatory approval and such requirement would delay our introduction of MRX34 or other product candidates into the market. These studies could also be required at any time after regulatory approval of a product candidate. Absence of long-term data may also limit the approved uses of a product, if any, to short-term use. MRX34 or any other product candidate may prove to be unsafe for human use, which would materially harm our business.

Certain oligonucleotide therapeutics and liposomal drug delivery products have shown injection site reactions, infusion reactions and pro-inflammatory effects and may also lead to impairment of organ function, including kidney or

liver function. There is a risk that our current and future product candidates may induce similar adverse events, or require pre- or co-administration of other drugs to minimize such effects, which pre- or co-administration might adversely affect the benefits of our product or add additional side effects to the treatment regimens. Results of our clinical trials could reveal a high and unacceptable severity and prevalence of these or other side effects. In such an event, our trials could be suspended or terminated and the FDA or comparable foreign regulatory authorities could order us to cease further development of or deny approval of our product candidates for any or all indications. Drug-related side effects could affect patient recruitment or the ability of enrolled patients to complete the trial or result in potential product liability claims. Any of these occurrences may significantly harm our business, financial condition, results of operations and prospects significantly.

Our product candidates may cause undesirable side effects or have other properties that could delay or prevent their regulatory approval, limit the commercial profile of an approved label, or result in significant negative consequences following marketing approval, if any.

As with many pharmaceutical products and product candidates under development, MRX34 or our other potential product candidates may produce undesirable side effects or adverse reactions or events. In the event we or others identify undesirable side effects caused by one of our product candidates, any of the following adverse events could occur:

- we may be required, or we may decide, to halt or delay further clinical development of our product candidates;
- the FDA or comparable foreign regulatory authorities could order us to cease further development of or deny approval of our product candidates for any or all indications; or
- product-related side effects could affect patient recruitment or the ability of enrolled patients to complete the trial or result in potential product liability claims.

If MRX34 or our other potential product candidates receives marketing approval, and we or others later identify undesirable side effects caused by such products, a number of potentially significant negative consequences could result, including:

- regulatory authorities may withdraw their approval of the product;
- we may be required to recall a product or change the way such product is administered to patients;
- additional restrictions may be imposed on the marketing of the particular product or the manufacturing processes for the product or any component thereof;
- regulatory authorities may require the addition of labeling statements, such as a “black box” warning or a contraindication;
- we may be required to implement a REMS or create a Medication Guide outlining the risks of such side effects for distribution to patients;
- we could be sued and held liable for harm caused to patients;
- the product may become less competitive; and
- our reputation may suffer.

Any of the foregoing events could prevent us from achieving or maintaining market acceptance of the particular product candidate, if approved, and result in the loss of significant revenues to us, which would materially and adversely affect our results of operations and business.

Our clinical drug development program may not uncover all possible adverse events that patients who take MRX34 or other product candidates may experience. The number of subjects exposed to MRX34 or other product candidates and the average exposure time in the clinical development program may be inadequate to detect rare adverse events, or chance findings, that may only be detected once the product is administered to more patients and for greater periods of time.

Clinical trials by their nature utilize a sample of the potential patient population. However, with a limited number of subjects and limited duration of exposure, we cannot be fully assured that rare and severe side effects of MRX34 or other product candidates will be uncovered. Such rare and severe side effects may only be uncovered with a significantly larger number of patients exposed to the drug. If such safety problems occur or are identified after MRX34 or another product candidate reaches the market, the FDA may require that we amend the labeling of the product or recall the product, or may even withdraw approval for the product.

We face potential product liability, and, if successful claims are brought against us, we may incur substantial liability and costs. If the use or misuse of our product candidates harms patients, or is perceived to harm patients even when such harm is unrelated to our product candidates, our regulatory approvals could be revoked or otherwise negatively impacted and we could be subject to costly and damaging product liability claims. If we are unable to obtain adequate insurance or are required to pay for liabilities resulting from a claim excluded from, or beyond the limits of, our insurance coverage, a material liability claim could adversely affect our financial condition.

The use or misuse of our product candidates in clinical trials and the sale of any products for which we obtain marketing approval exposes us to the risk of product liability claims. Product liability claims might be brought against us by consumers, healthcare providers, pharmaceutical companies or others selling or otherwise coming into contact with our products. There is a risk that our product candidates may induce adverse events. If we cannot successfully defend against product liability claims, we could incur substantial liability and costs. Certain oligonucleotide therapeutics and liposomal drug delivery products have shown injection site reactions, infusion reactions, and pro-inflammatory effects, and may also lead to organ dysfunction, including impairment of kidney or liver function. There is a risk that our future product candidates may induce similar adverse events. Patients with the diseases targeted by our product candidates are often already in severe and advanced stages of disease and have both known and unknown significant pre-existing and potentially life-threatening health risks. During the course of treatment, patients may suffer adverse events, including death, for reasons that may be related to our product candidates. Such events could subject us to costly litigation, require us to pay substantial amounts of money to injured patients, delay, negatively impact or end our opportunity to receive or maintain regulatory approval to market our products, or require us to suspend or abandon our commercialization efforts. Even in a circumstance in which we do not believe that an adverse event is related to our products, the investigation into the circumstance may be time-consuming or inconclusive. These investigations may interrupt our sales efforts, delay our regulatory approval process in other countries, or impact and limit the type of regulatory approvals our product candidates receive or maintain. As a result of these factors, a product liability claim, even if successfully defended, could have a material adverse effect on our business, financial condition or results of operations.

Although we have product liability insurance that we feel is appropriate for our stage of development, which covers our clinical trials in the United States, for up to $1 million per occurrence, up to an aggregate limit of $5 million, our insurance may be insufficient to reimburse us for any expenses or losses we may suffer, and we will be required to increase our product liability insurance coverage for our advanced clinical trials that we plan to initiate. We have obtained an additional product liability insurance policy for our clinical trials in the Republic of Korea. If and when we obtain marketing approval for product candidates, we intend to expand our insurance coverage to include the sale of commercial products. We do not know whether we will be able to continue to obtain product liability coverage and obtain expanded coverage if we require it, in sufficient amounts to protect us against losses due to liability, on acceptable terms, or at all. We may not have sufficient resources to pay for any liabilities resulting from a claim excluded from, or beyond the limits of, our insurance coverage. Where we have provided indemnities in favor of third parties under our agreements with them, there is also a risk that these third parties could incur liability and bring a claim under such indemnities. An individual may bring a product liability claim against us alleging that one of our product candidates or products causes, or is claimed to have caused, an injury or is found to be unsuitable for consumer use. Any such product liability claims may include allegations of defects in manufacturing, defects in design, a failure to warn of dangers

inherent in the product, negligence, strict liability, and a breach of warranties. Claims could also be asserted under state consumer protection acts. Any product liability claim brought against us, with or without merit, could result in:

- withdrawal of clinical trial volunteers, investigators, patients or trial sites or limitations on approved indications;
- the inability to commercialize, or if commercialized, decreased demand for, our product candidates;
- if commercialized, product recalls, withdrawals or labeling, marketing or promotional restrictions or the need for product modification;
- initiation of investigations by regulators;
- loss of revenues;
- substantial costs of litigation, including monetary awards to patients or other claimants;
- liabilities that substantially exceed our product liability insurance, which we would then be required to pay ourselves;
- an increase in our product liability insurance rates or the inability to maintain insurance coverage in the future on acceptable terms, if at all;
- the diversion of management's attention from our business; and
- damage to our reputation and the reputation of our products and our technology.

Product liability claims may subject us to the foregoing and other risks, which could have a material adverse effect on our business, financial condition, results of operations and prospects.

Currently, our product candidates are expensive to produce and are expensive relative to presently-marketed therapeutics targeting similar indications.

To date, our proposed product candidates have only been manufactured at a scale that is adequate to supply our research activities and early-stage clinical trials. As with many companies conducting Phase 1 and Phase 2 clinical trials or preclinical studies on product candidates, the current cost of each treatment is expensive relative to presently-marketed therapeutics targeting similar indications. We cannot assure you that we will be able to scale the manufacturing of our products during future clinical trials or commercialization in order to achieve a treatment price that would allow for commercial acceptance. In the event our product candidates cannot be manufactured in sufficient commercial quantities at a competitive price, our future prospects could be significantly impacted and our financial prospects would be materially harmed.

Even if a product candidate does obtain regulatory approval, that product candidate may never achieve market acceptance or commercial success.

Even if we obtain FDA or other regulatory approvals, and are able to launch MRX34 or any other product candidate commercially, the product candidate may not achieve market acceptance among physicians, patients, patient advocacy groups and third-party payors and, ultimately, may not be commercially successful. Market acceptance of any product candidate for which we receive approval depends on a number of factors, including:

- the efficacy and safety of the product candidate as demonstrated in clinical trials;
- the clinical indications for which the product candidate is approved;

- acceptance by physicians, patients, operators of treatment facilities and parties responsible for reimbursement of the product candidate as a safe and effective treatment;
- the potential and perceived advantages of the product candidate, including the cost of treatment and benefits over alternative treatments;
- the safety of the product candidate seen in a broader patient group, including use outside the approved indications;
- the cost of treatment in relation to alternative treatments;
- the availability of adequate reimbursement and pricing by third-party payors and government authorities;
- relative convenience and ease of administration;
- the tolerance of the products by patients, including prevalence and severity of adverse side effects;
- the availability of the product and the ability to meet market demand; and
- the effectiveness of our sales and marketing efforts.

Any failure by MRX34 or any other product candidate that obtains regulatory approval to achieve market acceptance or commercial success would adversely affect our financial results.

Risks Related to Our Reliance on Third Parties

We rely on third parties to conduct some of our nonclinical and all of our clinical trials. If these third parties do not successfully carry out their contractual duties or meet expected deadlines, we may be unable to obtain regulatory approval for or commercialize any of our product candidates.

Although we conduct certain nonclinical studies, we currently do not have the ability to independently conduct nonclinical studies that comply with the regulatory requirements known as good laboratory practice, or GLP, requirements. We also do not currently have the ability to independently conduct any clinical trials. The FDA and regulatory authorities in other jurisdictions require us to comply with regulations and standards, commonly referred to as current good clinical practice, or GCP, requirements for conducting, monitoring, recording and reporting the results of clinical trials, in order to ensure that the data and results are scientifically credible and accurate and that the trial subjects are adequately informed of the potential risks of participating in clinical trials. We rely on medical institutions, clinical investigators, contract laboratories and other third parties, such as CROs, to conduct GLP-compliant nonclinical studies and GCP-compliant clinical trials on our product candidates properly and on time. While we will have agreements governing their activities, we control only certain aspects of their activities and have limited influence over their actual performance. The third parties with whom we contract for execution of our GLP nonclinical studies and our GCP clinical trials play a significant role in the conduct of these studies and trials and the subsequent collection and analysis of data. These third parties are not our employees and, except for restrictions imposed by our contracts with such third parties, we have limited ability to control the amount or timing of resources that they devote to our programs. Although we rely on these third parties to conduct our GLP-compliant preclinical and nonclinical studies and GCP-compliant clinical trials, we remain responsible for ensuring that each of our GLP preclinical and nonclinical studies and GCP clinical trials is conducted in accordance with its investigational plan and protocol and applicable laws and regulations, and our reliance on the CROs does not relieve us of our regulatory responsibilities.

Many of the third parties with whom we contract may also have relationships with other commercial entities, including our competitors, for whom they may also be conducting clinical trials or other drug development activities that could harm our competitive position. If the third parties conducting our GLP preclinical or nonclinical studies or our clinical trials do not perform their contractual duties or obligations, experience work stoppages, do not meet expected deadlines, terminate their agreements with us or need to be replaced, or if the quality or accuracy of the clinical data they

obtain is compromised due to their failure to adhere to our clinical trial protocols or to GCPs, or for any other reason, we may need to enter into new arrangements with alternative third parties. This could be difficult, costly or impossible, and our nonclinical studies or clinical trials may need to be extended, delayed, terminated or repeated. As a result we may not be able to obtain regulatory approval in a timely fashion, or at all, for the applicable product candidate, our financial results and the commercial prospects for our product candidates would be harmed, our costs could increase, and our ability to generate revenues could be delayed.

We rely on a limited number of third-party contract manufacturing organizations to manufacture and supply MRX34 and other product candidates for us. If our supplier or manufacturer fails to perform adequately or fulfill our needs, or if these agreements are terminated by the third parties, we may be required to incur significant costs and devote significant efforts to find new suppliers or manufacturers. We may also face delays in the development and commercialization of our product candidates.

We do not currently independently conduct manufacturing activities for our product candidates, including MRX34. We rely upon single source third-party contract manufacturing organizations to manufacture and supply our product candidates. We currently have a relationship with two suppliers for clinical supply of the drug substance for our miR-34 mimic. Polymun Scientific Immunbiologische Forschung GmbH, or Polymun, located in Austria, is the exclusive manufacturer of our MRX34 drug product. Further, we rely on our contract manufacturers to manage the supply chain for the raw materials used in the manufacture of the drug substance and drug product.

Any manufacturers of the drug substance and drug product for our product candidates must comply with current good manufacturing practice, or cGMP, requirements enforced by the FDA through its facilities inspection program. These requirements include, among other things, quality control, quality assurance and the maintenance of records and documentation. Manufacturers of our component materials may be unable to comply with these cGMP requirements and with other FDA, state and foreign regulatory requirements. We do not directly control the manufacturing process of, and are completely dependent on, our contract manufacturing partners for compliance with cGMPs. If our contract manufacturers cannot successfully manufacture material that conforms to our specifications and the strict regulatory requirements of the FDA or foreign regulatory agencies, they will not be able to secure and/or maintain regulatory approval for their manufacturing facilities. In addition, we have no direct control over the ability of our contract manufacturers to maintain adequate quality control, quality assurance and qualified personnel. The FDA or similar foreign regulatory agencies at any time may also implement new standards, or change their interpretation and enforcement of existing standards for manufacture, packaging or testing of products. We have little control over a manufacturer's compliance with these regulations and standards. However, a failure to comply with these requirements may result in fines and civil penalties, suspension of production, suspension or delay in product approval, product seizure or recall, or withdrawal of product approval. If the safety of any product supplied is compromised due to our manufacturer's failure to adhere to applicable laws or for other reasons, we may not be able to obtain regulatory approval for or successfully commercialize our products, and we may be held liable for any injuries sustained as a result. In addition, if the FDA or a comparable foreign regulatory agency does not approve our contract manufacturer's facilities for the manufacture of our product candidates or if it withdraws its approval in the future, we may need to find alternative manufacturing facilities, which would negatively impact our ability to develop, obtain regulatory approval for, or market our product candidates, if approved. Any of these factors could cause a delay of clinical trials, regulatory submissions, approvals or commercialization of our product candidates or entail higher costs or impair our reputation.

The manufacture of pharmaceutical products in compliance with cGMP regulations requires significant expertise and capital investment, including the development of advanced manufacturing techniques and process controls. Manufacturers of pharmaceutical products often encounter difficulties in production, including difficulties with production costs and yields, quality control, including stability of the product candidate and quality assurance testing, or shortages of qualified personnel. If our manufacturers were to encounter any of these difficulties or otherwise fail to comply with their obligations to us or under applicable regulations, our ability to provide study materials in our nonclinical studies and clinical trials would be jeopardized. Any delay or interruption in the supply of nonclinical study or clinical trial materials could delay the completion of our nonclinical studies and clinical trials, increase the costs associated with maintaining our nonclinical study and clinical trial programs and, depending upon the period of delay, require us to conduct nonclinical studies, commence new trials at significant additional expense or terminate the studies and trials completely.

We currently believe that our third party suppliers have the necessary expertise to produce our MRX34 drug substance and drug product in sufficient quantity and of acceptable quality to support our development program through at least Phase 3 clinical trials and possibly through commercialization of MRX34. However, our current agreements with our suppliers do not provide for the entire supply of the drug necessary for additional clinical trials or for full-scale commercialization. In the event that we and our suppliers cannot agree to the terms and conditions for them to provide some or all of our clinical and commercial drug supply needs, or if our suppliers terminate their agreements with us in response to a breach by us or any other reason permitted under our agreements, we would not be able to manufacture the drug on a commercial scale until a qualified alternative supplier is identified, which could also delay the development of, and impair our ability to commercialize, our product candidates. Any supplier would be required to obtain regulatory approval of their manufacturing facilities, processes and quality systems before engaging in the commercial manufacture of a pharmaceutical product. Due to the complexity of the processes used to manufacture pharmaceutical products and product candidates, any potential third-party manufacturer may be unable to continue to pass or initially pass federal, state or international regulatory inspections in a cost-effective manner.

Although we believe that appropriate alternative sources of supply exist for each of our current product candidates, the number of third-party suppliers with the necessary manufacturing and regulatory expertise and facilities is limited, and it could be expensive and take a significant amount of time to arrange for alternative suppliers, which could have a material adverse effect on our business. New suppliers of any drug would be required to qualify under applicable regulatory requirements and would need to have sufficient rights under applicable intellectual property laws to the method of manufacturing such ingredients. Obtaining the necessary FDA approvals or other qualifications under applicable regulatory requirements and ensuring non-infringement of third- party intellectual property rights could result in a significant interruption of supply and could require the new manufacturer to bear significant additional costs which may be passed on to us.

The failure of third-party manufacturers or suppliers to perform adequately or the termination of our arrangements with any of them may negatively and adversely affect our business.

Reliance on third-party manufacturers entails risks to which we would not be subject if we manufactured the product candidates ourselves, including:

- the inability to meet any product specifications and quality requirements consistently;
- a delay or inability to procure or expand sufficient manufacturing capacity;
- capacity related to the scale-up of manufacturing;
- manufacturing and product quality issues related to scale-up of manufacturing;
- costs and validation of new equipment and facilities required for scale-up;
- a failure to comply with cGMP and similar foreign standards;
- operations of our third-party manufacturers or suppliers could be disrupted by conditions unrelated to our business or operations, including the bankruptcy of the manufacturer or supplier;
- carrier disruptions or increased costs that are beyond our control;
- the failure of third parties involved in the transportation, storage and distribution of our products, including the failure to deliver products under specified storage conditions and in a timely manner; and
- the possibility that our contract manufacturer, or third parties with access to their facilities, will have access to and may appropriate our trade secrets or other proprietary information.

Any of these events could lead to clinical trial delays or failure to obtain regulatory approval, or impact our ability to successfully commercialize future products. Some of these events could be the basis for FDA action, including injunction, recall, seizure or total or partial suspension of production.

We may not be able to develop or identify a technology that can effectively deliver our miR-34 mimic or any other of our microRNA-based product candidates to the intended diseased cells or tissues, and any failure in such delivery technology could adversely affect and delay the development of MRX34 and our other product candidates.

In connection with our Phase 1 clinical trial of MRX34, we have used a SMARTICLES liposomal formulation to facilitate delivery to tumors. SMARTICLES has demonstrated successful tumor delivery of our miR-34 mimic in multiple mouse models of liver cancer, but we cannot be certain that the SMARTICLES technology will be capable of delivering adequate levels of our miR-34 mimic to tumors in patients to produce a therapeutic response. While we are continuing to evaluate the use of SMARTICLES in different indications, and additional delivery technologies that might enable us to target specific cancer cells with our product candidates, we cannot be certain whether we will be successful in developing such alternative delivery mechanisms. Our failure to effectively deliver any of our product candidates to the intended diseased cells or tissues could adversely affect and delay the development of our product candidates.

We currently have no sales and marketing staff or distribution organization. If we are unable to develop a sales and marketing and distribution capability on our own or through third parties, we will not be successful in commercializing our future products.

We currently have no sales, marketing or distribution capabilities or experience. To achieve commercial success for any approved product candidate, we must either develop a sales, marketing and distribution organization or outsource these functions to third parties. If we rely on third parties for marketing and distributing our approved products, any revenue we receive will depend upon the efforts of third parties, which may not be successful and are only partially within our control and our product revenue may be lower than if we directly marketed or sold our products. If we are unable to enter into arrangements with third parties to sell, market and distribute product candidates for which we have received regulatory approval on acceptable terms or at all, we will need to market these products ourselves. This is likely to be expensive and logistically difficult, as it would require us to build our own sales, marketing and distribution capacity. We have no experience in this area, and if such efforts were necessary, we may not be able to successfully commercialize our future products. If we are not successful in commercializing our future products, either on our own or through third parties, any future product revenue will be materially and adversely affected.

We may attempt to form collaborations in the future with respect to our product candidates, but we may not be able to do so, which may cause us to alter our development and commercialization plans.

We may attempt to form strategic alliances, create joint ventures or collaborations or enter into licensing arrangements with third parties with respect to our programs that we believe will complement or augment our existing business. For example, we may attempt to find a strategic partner for the development and/or commercialization of MRX34. We may face significant competition in seeking appropriate strategic partners, and the negotiation process to secure appropriate terms is time-consuming and complex. We may not be successful in our efforts to establish such a strategic partnership for any product candidates and programs on terms that are acceptable to us, or at all. This may be because our product candidates and programs may be deemed to be at too early of a stage of development for collaborative effort, our research and development pipeline may be viewed as insufficient, the competitive or intellectual property landscape may be viewed as too intense or risky, and/or third parties may not view our product candidates and programs as having sufficient potential for commercialization, including the likelihood of an adequate safety and efficacy profile.

Any delays in identifying suitable collaborators and entering into agreements to develop and/or commercialize our product candidates could delay the development or commercialization of our product candidates, which may reduce their competitiveness even if they reach the market. Absent a collaboration partner, we would need to undertake development and/or commercialization activities at our own expense. If we elect to fund and undertake development and/or commercialization activities on our own, we may need to obtain additional expertise and additional capital, which

may not be available to us on acceptable terms or at all. If we are unable to do so, we may not be able to develop our product candidates or bring them to market and our business may be materially and adversely affected.

We may be unable to realize the potential benefits of any collaboration.

Even if we are successful in entering into a collaboration with respect to the development and/or commercialization of one or more product candidates, there is no guarantee that the collaboration will be successful. Collaborations may pose a number of risks, including:

- collaborators often have significant discretion in determining the efforts and resources that they will apply to the collaboration, and may not commit sufficient resources to the development, marketing or commercialization of the product or products that are subject to the collaboration;
- collaborators may not perform their obligations as expected;
- any such collaboration may significantly limit our share of potential future profits from the associated program, and may require us to relinquish potentially valuable rights to our current product candidates, potential products or proprietary technologies or grant licenses on terms that are not favorable to us;
- collaborators may cease to devote resources to the development or commercialization of our product candidates if the collaborators view our product candidates as competitive with their own products or product candidates;
- disagreements with collaborators, including disagreements over proprietary rights, contract interpretation or the course of development, might cause delays or termination of the development or commercialization of product candidates, and might result in legal proceedings, which would be time- consuming, distracting and expensive;
- collaborators may be impacted by changes in their strategic focus or available funding, or business combinations involving them, which could cause them to divert resources away from the collaboration;
- collaborators may infringe the intellectual property rights of third parties, which may expose us to litigation and potential liability;
- the collaborations may not result in us achieving revenues to justify such transactions; and
- collaborations may be terminated and, if terminated, may result in a need for us to raise additional capital to pursue further development or commercialization of the applicable product candidate.

As a result, a collaboration may not result in the successful development or commercialization of our product candidates.

Reliance on government funding for our programs may add uncertainty to our research and commercialization efforts with respect to those programs that are tied to such funding and may impose requirements that limit our ability to take certain actions, increase the costs of commercialization and production of product candidates developed under those programs and subject us to potential financial penalties, which could materially and adversely affect our business, financial condition and results of operations.

During the course of our development of our product candidates, we have been funded in significant part through federal and state grants, including but not limited to the substantial funding we have received from the Texas Emerging Technology Fund and the Cancer Prevention and Research Institute of Texas, or CPRIT. In addition to the funding we have received to date, we have applied and intend to continue to apply for federal and state grants to receive additional funding in the future. Contracts and grants funded by the U.S. government, state governments and their related agencies, including our contracts with the State of Texas pertaining to funds we have already received, include

provisions that reflect the government's substantial rights and remedies, many of which are not typically found in commercial contracts, including powers of the government to:

- require repayment of all or a portion of the grant proceeds, in certain cases with interest, in the event we violate certain covenants pertaining to various matters that include any potential relocation outside of the State of Texas, failure to achieve certain milestones or to comply with terms relating to use of grant proceeds, or failure to comply with certain laws;
- terminate agreements, in whole or in part, for any reason or no reason;
- reduce or modify the government's obligations under such agreements without the consent of the other party;
- claim rights, including intellectual property rights, in products and data developed under such agreements;
- audit contract-related costs and fees, including allocated indirect costs;
- suspend the contractor or grantee from receiving new contracts pending resolution of alleged violations of procurement laws or regulations;
- impose U.S. manufacturing requirements for products that embody inventions conceived or first reduced to practice under such agreements;
- impose qualifications for the engagement of manufacturers, suppliers and other contractors as well as other criteria for reimbursements;
- suspend or debar the contractor or grantee from doing future business with the government;
- control and potentially prohibit the export of products;
- pursue criminal or civil remedies under the False Claims Act, False Statements Act and similar remedy provisions specific to government agreements; and
- limit the government's financial liability to amounts appropriated by the U.S. Congress on a fiscal-year basis, thereby leaving some uncertainty about the future availability of funding for a program even after it has been funded for an initial period.

In addition to those powers set forth above, the government funding we may receive could also impose requirements to make payments based upon sales of our products in the future. For example, under the terms of our 2010 award from CPRIT, we are required to pay CPRIT a portion of our revenues from sales of certain products by us, or received from our licensees or sublicensees, at a percentage in the low single digits until the aggregate amount of such payments equals a specified multiple of the grant amount, and thereafter at a rate of less than one percent, subject to our right, under certain circumstances, to make a one-time payment in a specified amount to CPRIT to buy out such payment obligations. See "Business—Key Partnerships and Licenses" for a description of this CPRIT agreement, which includes a description of our obligations to make royalty payments.

We may not have the right to prohibit the U.S. government from using certain technologies developed by us, and we may not be able to prohibit third-party companies, including our competitors, from using those technologies in providing products and services to the U.S. government. The U.S. government generally takes the position that it has the right to royalty-free use of technologies that are developed under U.S. government contracts. These and other provisions of government grants may also apply to intellectual property we license now or in the future.

In addition, government contracts and grants normally contain additional requirements that may increase our costs of doing business, reduce our profits, and expose us to liability for failure to comply with these terms and conditions. These requirements include, for example:

- specialized accounting systems unique to government contracts and grants;
- mandatory financial audits and potential liability for price adjustments or recoupment of government funds after such funds have been spent;
- public disclosures of certain contract and grant information, which may enable competitors to gain insights into our research program; and
- mandatory socioeconomic compliance requirements, including labor standards, non-discrimination and affirmative action programs and environmental compliance requirements.

If we fail to maintain compliance with any such requirements that may apply to us now or in the future, we may be subject to potential liability and to termination of our contracts.

Our business involves the use of hazardous materials and we and our third- party manufacturers must comply with environmental laws and regulations, which may be expensive and restrict how we do business.

Our third-party manufacturers' activities and our own activities involve the controlled storage, use and disposal of hazardous materials, including the components of our pharmaceutical product candidates, test samples and reagents, biological materials and other hazardous compounds. We and our manufacturers are subject to federal, state, local and foreign laws and regulations governing the use, generation, manufacture, storage, handling and disposal of these hazardous materials. We currently carry no insurance specifically covering environmental claims relating to the use of hazardous materials. Although we believe that our safety procedures for handling and disposing of these materials and waste products comply with the standards prescribed by these laws and regulations, we cannot eliminate the risk of accidental injury or contamination from the use, storage, handling or disposal of hazardous materials. In the event of an accident, state or federal or other applicable authorities may curtail our use of these materials and/or interrupt our business operations. In addition, if an accident or environmental discharge occurs, or if we discover contamination caused by prior operations, including by prior owners and operators of properties we acquire, we could be liable for cleanup obligations, damages and fines. If such unexpected costs are substantial, this could significantly harm our financial condition and results of operations.

Risks Related to Administrative, Organizational and Commercial Operations and Growth

We will need to increase the size of our organization, and we may experience difficulties in managing growth.

As of March 31, 2016, we had 33 employees. We may need to expand our managerial, operational, financial and other resources in order to manage our operations and clinical trials, continue our development activities and commercialize MRX34 or other product candidates. Our management and personnel, systems and facilities currently in place are likely not adequate to support this future growth. Also, our management may need to divert a disproportionate amount of its attention away from our day-to-day activities and devote a substantial amount of time to managing these growth activities. We may not be able to effectively manage the expansion of our operations, which may result in weaknesses in our infrastructure and give rise to operational mistakes, loss of business opportunities, loss of employees and reduced productivity among remaining employees. Our expected growth could require significant capital expenditures and may divert financial resources from other projects, such as the development of additional product candidates. Our need to effectively execute our business strategy requires that we:

- manage our Phase 1 clinical trial, which is being conducted at multiple trial sites, as well as manage any other clinical trials in the future;

- manage our internal development efforts effectively while carrying out our contractual obligations to licensors, contractors, government agencies, any future collaborators and other third parties;

- continue to improve our operational, financial and management controls, reporting systems and procedures; and

- identify, recruit, maintain, motivate and integrate additional employees.

If we are unable to expand our managerial, operational, financial and other resources to the extent required to manage our development and commercialization activities, our business will be materially adversely affected.

We face substantial competition and our competitors may discover, develop or commercialize products faster or more successfully than us.

The development and commercialization of new drug products is highly competitive. We face competition from major pharmaceutical companies, specialty pharmaceutical companies, biotechnology companies, universities and other research institutions worldwide with respect to MRX34 and other product candidates that we may seek to develop or commercialize in the future. Our competitors may succeed in developing, acquiring or licensing technologies and drug products that are more effective or less costly than MRX34 or any other product candidates that we are currently developing or that we may develop, which could render our product candidates obsolete and noncompetitive.

There are a number of pharmaceutical and biotechnology companies that currently market and sell products or are pursuing the development of product candidates for the treatment of solid tumors. The most common treatments for solid tumors are various chemotherapeutic agents, radiation therapy and certain targeted therapies, including monoclonal antibodies such as Avastin®, Erbitux®, Herceptin® and Vectibix®. Small molecules, such as Nexavar, Sutent® and Tarceva®, are also indicated for the treatment of solid tumors.

In addition to the competition we face from alternative therapies for the diseases we intend to target with our product candidates, we are also aware of several companies that are also working specifically to develop microRNA therapeutics, including miRagen Therapeutics, Inc., Regulus Therapeutics, Inc. and Santaris Pharma A/S (now Roche). Many of our competitors have substantially greater financial, technical and other resources, such as larger research and development staff and experienced marketing and manufacturing organizations. Insurers and other third-party payors may also encourage the use of generic products. For example, if MRX34 is approved, it may be priced at a significant premium over other competitive products. This may make it difficult for MRX34 or any other future products to compete with these products.

If our competitors obtain marketing approval from the FDA or comparable foreign regulatory authorities for their product candidates more rapidly than us, it could result in our competitors establishing a strong market position before we are able to enter the market.

Many of our competitors have materially greater name recognition and financial, manufacturing, marketing, research and drug development resources than we do. Additional mergers and acquisitions in the biotechnology and pharmaceutical industries may result in even more resources being concentrated in our competitors. Large pharmaceutical companies in particular have extensive expertise in preclinical, nonclinical and clinical testing and in obtaining regulatory approvals for drugs. In addition, academic institutions, government agencies, and other public and private organizations conducting research may seek patent protection with respect to potentially competitive products or technologies. These organizations may also establish exclusive collaborative or licensing relationships with our competitors. Failure of MRX34 or other product candidates to effectively compete against established treatment options or in the future with new products currently in development would harm our business, financial condition, results of operations and prospects.

We may engage in strategic transactions that could impact our liquidity, increase our expenses and present significant distractions to our management.

From time to time, we may consider strategic transactions, such as acquisitions of companies, asset purchases, and out-licensing or in-licensing of products, product candidates or technologies. Additional potential transactions that we may consider include a variety of different business arrangements, including spin-offs, strategic partnerships, joint ventures, restructurings, divestitures, business combinations and investments. Any such transaction may require us to incur non-recurring or other charges, may increase our near- and long-term expenditures and may pose significant integration challenges or disrupt our management or business, which could adversely affect our operations and financial results. For example, these transactions may entail numerous operational and financial risks, including:

- exposure to unknown liabilities;
- disruption of our business and diversion of our management's time and attention in order to develop acquired products, product candidates or technologies;
- incurrence of substantial debt or dilutive issuances of equity securities to pay for acquisitions;
- higher-than-expected acquisition and integration costs;
- write-downs of assets or goodwill or impairment charges;
- increased amortization expenses;
- difficulty and cost in combining the operations and personnel of any acquired businesses with our operations and personnel;
- impairment of relationships with key suppliers or customers of any acquired businesses due to changes in management and ownership; and
- inability to retain key employees of any acquired businesses.

Accordingly, although there can be no assurance that we will undertake or successfully complete any transactions of the nature described above, any transactions that we do complete may be subject to the foregoing or other risks, could have a material adverse effect on our business, financial condition, results of operations and prospects.

We are highly dependent on the services of our President and Chief Executive Officer, Paul Lammers, M.D., M.Sc., and our ability to attract and retain qualified personnel.

We may not be able to attract or retain qualified management and scientific and clinical personnel in the future due to the intense competition for qualified personnel among biotechnology, pharmaceutical and other businesses. Our industry has experienced a high rate of turnover of management and scientific personnel in recent years. If we are not able to attract, retain and motivate necessary personnel to accomplish our business objectives, we may experience constraints that will significantly impede the achievement of our development objectives, our ability to raise additional capital and our ability to implement our business strategy.

We are highly dependent on the principal members of our management and scientific staff. The loss of service of any of our management and key scientific staff could harm our business, particularly our President and Chief Executive Officer, Dr. Lammers. Due to our limited resources, we may not be able to effectively attract and recruit additional qualified personnel. If we are not able to retain our management, particularly our President and Chief Executive Officer, Dr. Lammers, and to attract, on acceptable terms, additional qualified personnel necessary for the continued development of our business, we may not be able to sustain our operations or grow. Although we have executed employment agreements with each member of our current executive management team, including Dr. Lammers, we may not be able to retain their services as expected.

In addition, we have scientific and clinical advisors who assist us in formulating our product development and clinical strategies. These advisors are not our employees and may have commitments to, or consulting or advisory contracts with, other entities that may limit their availability to us, or may have arrangements with other companies to assist in the development of products that may compete with ours.

Our internal computer systems, or those of our CROs or other contractors or consultants, may fail or suffer security breaches, which could result in a material disruption of our product development programs.

Despite the implementation of security measures, our internal computer systems and those of our CROs and other contractors and consultants are vulnerable to damage from computer viruses, unauthorized access, natural disasters, terrorism, war and telecommunication and electrical failures. While we have not experienced any such system failure, accident or security breach to date, if such an event were to occur and cause interruptions in our operations, it could result in a material disruption of our programs. For example, the loss of clinical trial data from completed or ongoing clinical trials for any of our product candidates could result in delays in our regulatory approval efforts and significantly increase our costs to recover or reproduce the data. To the extent that any disruption or security breach results in a loss of or damage to our data or applications, or inappropriate disclosure of confidential or proprietary information, including the confidential medical information of clinical trial participants, we could incur liability and the further development of our product candidates could be delayed.

Our employees, independent contractors, principal investigators, CROs, consultants and vendors may engage in misconduct or other improper activities, including noncompliance with regulatory standards and requirements.

We are exposed to the risk that employees, independent contractors, principal investigators, CROs, consultants and vendors may engage in fraudulent or other illegal activity. Misconduct by these parties could include intentional, reckless and/or negligent conduct or disclosure of unauthorized activities to us that violates: (i) FDA regulations, including those laws requiring the reporting of true, complete and accurate information to the FDA; (ii) manufacturing standards; (iii) federal and state healthcare fraud and abuse laws and regulations; or (iv) laws that require the true, complete and accurate information or data. Specifically, sales, marketing and business arrangements in the healthcare industry are subject to extensive laws and regulations intended to prevent fraud, kickbacks, self-dealing and other abusive practices. These laws and regulations may restrict or prohibit a wide range of pricing, discounting, marketing and promotion, sales commission, customer incentive programs and other business arrangements. Activities subject to these laws also involve the improper use of information obtained in the course of clinical trials, which could result in regulatory sanctions and serious harm to our reputation. It is not always possible to identify and deter misconduct by our employees and other third parties, and the precautions we take to detect and prevent this activity may not be effective in controlling unknown or unmanaged risks or losses or in protecting us from governmental investigations or other actions or lawsuits stemming from a failure to be in compliance with such laws or regulations. If any such actions are instituted against us, and we are not successful in defending ourselves or asserting our rights, those actions could have a significant impact on our business, including the imposition of civil, criminal and administrative penalties, damages, monetary fines, possible exclusion from participation in Medicare, Medicaid and other federal healthcare programs, contractual damages, reputational harm, diminished profits and future earnings, and curtailment of our operations, any of which could adversely affect our ability to operate our business and our results of operations.

Requirements associated with being a public company have increased and will continue to increase our costs significantly, as well as divert significant company resources and management attention.

Prior to our IPO, we were not subject to the reporting requirements of the Securities Exchange Act of 1934, as amended, or the Exchange Act, or the other rules and regulations of the Securities and Exchange Commission, or SEC, or any securities exchange relating to public companies. We are working with our legal, independent accounting and financial advisors to identify those areas in which changes should be made to our financial and management control systems to manage our growth and our obligations as a public company. These areas include corporate governance, corporate control, disclosure controls and procedures and financial reporting and accounting systems. We have made, and will continue to make, changes in these and other areas. However, the expenses that will be required in order to operate as a public company are, and could continue to be, material, particularly after we cease to be an “emerging

growth company." Compliance with the various reporting and other requirements applicable to public companies will also require considerable time and attention of management. In addition, the changes we have made and make may not be sufficient to allow us to satisfy our obligations as a public company on a timely basis.

However, for as long as we remain an "emerging growth company" as defined in the Jumpstart our Business Startups Act, or the JOBS Act, we may take advantage of certain exemptions from various reporting requirements that are applicable to other public companies that are not "emerging growth companies," including not being required to comply with the auditor attestation requirements of Section 404 of the Sarbanes-Oxley Act, reduced disclosure obligations regarding executive compensation in our periodic reports and proxy statements and exemptions from the requirements of holding a nonbinding advisory vote on executive compensation and stockholder approval of any golden parachute payments not previously approved. We may take advantage of these reporting exemptions until we are no longer an "emerging growth company." Because the JOBS Act has only recently been enacted, it is not yet clear whether investors will accept the more limited disclosure requirements that we may be entitled to follow while we are an "emerging growth company." If they do not, we may end up electing to comply with disclosure requirements as if we were not an "emerging growth company," in which case we would incur the greater expenses associated with such disclosure requirements.

We will remain an "emerging growth company" for up to five years after the completion of our IPO, although if the market value of our common stock that is held by non-affiliates exceeds $700 million as of any September 30 before that time or if we have total annual gross revenues of $1 billion or more during any fiscal year before that time, we would cease to be an "emerging growth company" as of the end of that fiscal year, or if we issue more than $1 billion in non-convertible debt in a three-year period, we would cease to be an "emerging growth company" immediately.

In addition, being a public company could make it more difficult or more costly for us to obtain certain types of insurance, including directors' and officers' liability insurance, and we may be forced to accept reduced policy limits and coverage or incur substantially higher costs to obtain the same or similar coverage. The impact of these events could also make it more difficult for us to attract and retain qualified persons to serve on our board of directors, our board committees or as executive officers.

If we are not able to implement the requirements of Section 404 of the Sarbanes-Oxley Act of 2002 in a timely manner or with adequate compliance, we may be subject to sanctions by regulatory authorities.

Section 404 of the Sarbanes-Oxley Act of 2002 requires that we evaluate and determine the effectiveness of our internal controls over financial reporting and, beginning with our annual report for fiscal year 2016, provide a management report on the internal control over financial reporting. If we have a material weakness in our internal control over financial reporting, we may not detect errors on a timely basis and our financial statements may be materially misstated. We will be evaluating our internal controls systems to allow management to report on, and eventually allow our independent auditors to attest to, our internal controls. We will be performing the system and process evaluation and testing (and any necessary remediation) required to comply with the management certification and eventual auditor attestation requirements of Section 404 of the Sarbanes-Oxley Act of 2002. The aforementioned auditor attestation requirements will not apply to us until we are not an "emerging growth company."

To date, we have never conducted a review of our internal controls for the purpose of providing the reports required by these rules. We cannot be certain as to the timing of completion of our evaluation, testing and remediation actions or the impact of the same on our operations. If we are not able to implement the requirements of Section 404 in a timely manner or with adequate compliance, we may be subject to sanctions or investigation by regulatory authorities, such as the SEC or The NASDAQ Stock Market LLC, or NASDAQ. Any such action could adversely affect our financial results or investors' confidence in us and could cause our stock price to fall. Moreover, if we are not able to comply with the requirements of Section 404 in a timely manner, or if we or our independent registered public accounting firm identifies deficiencies in our internal controls that are deemed to be material weaknesses, we could be subject to sanctions or investigations by the SEC, NASDAQ or other regulatory authorities, which would entail expenditure of additional financial and management resources and could materially adversely affect our stock price. Inferior internal controls could also cause us to fail to meet our reporting obligations or cause investors to lose confidence in our reported financial information, which could have a negative effect on our stock price.

Our ability to utilize our net operating loss carryforwards and certain other tax attributes may be limited.

We have incurred substantial losses during our history and do not expect to become profitable in 2016 and may never achieve profitability. To the extent that we continue to generate taxable losses, unused losses will carry forward to offset future taxable income, if any, until such unused losses expire. We may be unable to use these losses to offset income before such unused losses expire. Under Sections 382 and 383 of the Internal Revenue Code of 1986, as amended, or the Code, if a corporation undergoes an "ownership change," generally defined as a greater than 50 percentage point change (by value) in its equity ownership over a three-year period, the corporation's ability to use its pre-change net operating loss, or NOL, carryforwards and other pre-change tax attributes to offset its post-change income or taxes may be further limited. We believe that we have experienced at least one ownership change in the past. We may also experience additional ownership changes as a result of subsequent shifts in our stock ownership, including as a result of our IPO. Accordingly, our ability to use our pre-change NOL carryforwards to offset U.S. federal taxable income may be subject to limitations, which could potentially result in increased future tax liability to us. For these reasons, we may not be able to utilize any or a material portion of our NOL carryforwards and other tax attributes.

If we seek and obtain approval to commercialize MRX34 outside of the United States, a variety of risks associated with international operations could materially adversely affect our business.

If MRX34 is approved for commercialization outside the United States, we will likely enter into agreements with third parties to market MRX34 outside the United States. We expect that we will be subject to additional risks related to entering into these international business relationships, including:

- different regulatory requirements for drug approvals in foreign countries;
- differing U.S. and foreign drug import and export rules;
- reduced protection for our intellectual property rights in foreign countries;
- existence of third party intellectual property rights of potential relevance to our business;
- unexpected changes in tariffs, trade barriers and regulatory requirements;
- different reimbursement systems;
- economic weakness, including inflation, or political instability in particular foreign economies and markets;
- compliance with tax, employment, immigration and labor laws for employees living or traveling abroad or with U.S. regulations that would apply to activities in such foreign jurisdictions, such as the Foreign Corrupt Practices Act;
- foreign taxes, including withholding of payroll taxes;
- foreign currency fluctuations, which could result in increased operating expenses and reduced revenues, and other obligations incident to doing business in another country;
- workforce uncertainty in countries where labor unrest is more common than in the United States;
- production shortages resulting from any events affecting raw material supply or manufacturing capabilities abroad;
- potential liability resulting from development work conducted by these distributors; and

· business interruptions resulting from geopolitical actions, including war and terrorism, or natural disasters.

We or the third parties upon whom we depend may be adversely affected by natural disasters and our business continuity and disaster recovery plans may not adequately protect us from a serious disaster.

Natural disasters could severely disrupt our operations, and have a material adverse effect on our business, financial condition and results of operations. If a natural disaster, power outage or other event occurred that prevented us from using all or a significant portion of our headquarters, that damaged critical infrastructure, such as our enterprise financial systems or manufacturing resource planning and enterprise quality systems, or that otherwise disrupted operations, it may be difficult or, in certain cases, impossible for us to continue our business for a substantial period of time. The disaster recovery and business continuity plans we have in place currently are limited and are unlikely to prove adequate in the event of a serious disaster or similar event. We may incur substantial expenses as a result of the limited nature of our disaster recovery and business continuity plans, which could have a material adverse effect on our business.

Furthermore, certain integral parties in our supply chain are geographically concentrated and operating from single sites, increasing their vulnerability to natural disasters or other sudden, unforeseen and severe adverse events. Although we believe there to be sufficient alternative suppliers in other geographic locations, if such an event were to affect such existing parties in our supply chain, it could have a material adverse effect on our business.

Risks Related to Intellectual Property

If we are unable to obtain and maintain sufficient patent protection for our technology and product candidates, or if the scope of the patent protection is not sufficiently broad, our competitors could develop and commercialize products similar or identical to ours, and our ability to successfully commercialize our product candidates may be adversely affected.

We rely upon a combination of patents, trade secret protection and confidentiality agreements to protect the intellectual property related to our technologies.

In particular, our success depends in large part on our ability to obtain and maintain patent protection in the United States and other countries with respect to our product candidates. If we do not adequately protect our intellectual property, competitors may be able to use our technologies and erode or negate any competitive advantage we may have, which could harm our business and ability to achieve profitability. To protect our proprietary position, we file patent applications in the United States and in limited jurisdictions abroad related to our product candidates and compounds in development that may become our product candidates. The patent application and approval process is expensive and time-consuming. We may not be able to file and prosecute all necessary or desirable patent applications at a reasonable cost or in a timely manner. We may also fail to identify patentable aspects of our research and development before it is too late to obtain patent protection.

The strength of patents in the biotechnology and pharmaceutical field involves complex legal and scientific questions and can be uncertain. This uncertainty includes changes to the patent laws through either legislative action to change statutory patent law or court action that may reinterpret existing law in ways affecting the scope or validity of issued patents. The patent applications that we own or in-license may fail to result in issued patents in the United States or in foreign countries in which we pursue protection with claims that cover our product candidates. There is no assurance that all of the potentially relevant prior art relating to our patents and patent applications has been found, which can invalidate a patent or prevent a patent from issuing from a pending patent application. Even if patents have issued, or do successfully issue, from patent applications that we own or license, third parties may challenge the validity, enforceability or scope thereof, which may result in such patents being narrowed, invalidated or held unenforceable. For example, patents granted by the European Patent Office, or EPO, may be challenged, also known as opposed, by any person within nine months from the publication of their grant. In May 2015, two separate and unidentified parties filed submissions before the EPO opposing a granted European patent related to MRX34, EP2302055 (the ’055 Patent), in-licensed to us from Asuragen. We have reviewed these submissions and have submitted our response. We are currently awaiting a response from the EPO. All of the claims of the ’055 Patent remain valid and in force during the opposition proceedings. It is not possible to predict the outcome of the opposition proceedings, for example whether the

patent will be maintained, limited in scope or whether the grant may be revoked. If the ’055 Patent is ultimately narrowed in scope or revoked during the opposition proceedings, the patent protection afforded by the ’055 Patent, and the extent of our exclusivity with respect to commercialization of MRX-34 in Europe could be materially impaired. Even if they are unchallenged, our patents may not adequately protect our product candidates, provide any competitive advantage or prevent others from designing around our claims. If the breadth or strength of protection provided by the patents and patent applications we hold, in-license or pursue with respect to our product candidates is threatened or insufficient, it could dissuade companies from collaborating with us to develop or undermine our ability to commercialize our product candidates and could have a material adverse effect on our business, financial condition, results of operations and prospects.

Currently, our patent portfolio includes over 10 issued U.S. patents and over 42 pending U.S. and ex-U.S. patent applications that we own, co-own, or have in-licensed from third parties, primarily focused on various aspects of microRNA therapeutics, including various microRNA mimics, and methods of use as microRNA related therapies. Within our patent portfolio, we are the sole owner of multiple U.S. and foreign patent applications related to microRNA therapies, including chemically modified versions of miR-34 not currently used in MRX34 (U.S. Patent No. 8,586,727) and other microRNAs mimics that are possible candidates for future product development as microRNA therapeutics. Further, our patent portfolio includes U.S. 7,960,359 and U.S. 8,563,708, both of which are related to miR-34 and are in-licensed from Asuragen. Specifically, U.S. 7,960,359 is related to use of a miR-34a mimic, for example MRX34, for reducing cell viability of human lung cancer cells, human cancerous T cells, human prostate cancer cells or human skin cancer cells. This patent is expected to expire in 2025. We also are the exclusive licensee with respect to MRX34 of US 9,006,206, which relates to use of miR-34 to treat a cancer associated with p53, and EP2126078, which relates to treatment of certain cancers that are also p53 negative. Both US 9,006,206 and EP 2126078 are co-owned by Rosetta Genomics and Yeda Research & Development. See “Business—Intellectual Property—Our Patent Portfolio” for a more detailed description of the patents we own or license covering our product candidates.

If the patent applications we hold or have in-licensed with respect to our programs or product candidates fail to issue, if their breadth or strength of protection is threatened, if we abandon or allow owned or in-licensed patents or patent applications that we are responsible for prosecuting to lapse, or if our owned and in-licensed patents and patent applications fail to provide meaningful exclusivity for our product candidates, it could dissuade companies from collaborating with us to develop product candidates, and threaten our ability to commercialize future products. We have multiple pending patent applications relating to our product candidates. We cannot offer any assurances about which, if any, patents will issue, the breadth of the claims of any such patent, should it issue, or whether any issued patents will be found invalid and/or unenforceable, will be interpreted narrowly or will be threatened by third parties. Any successful opposition to these patents or any other patents owned by or licensed to us could deprive us of rights necessary for the successful commercialization of any product candidates that we may develop.

Almost all of our patents and patent applications are entitled to effective filing dates prior to March 16, 2013. For U.S. patent applications in which patent claims are entitled to a priority date before March 16, 2013, an interference proceeding can be provoked by a third party, for example a competitor, or instituted by the U.S. Patent and Trademark Office, or the USPTO, to determine who was the first to invent any of the subject matter covered by those patent claims. An unfavorable outcome could require us to cease using the related technology or to attempt to license rights from the prevailing party. Our business could be harmed if the prevailing party does not offer us a license on commercially reasonable terms. Our participation in an interference proceeding may fail and, even if successful, may result in substantial costs and distract our management.

Further, if we encounter delays in our clinical trials or achieving regulatory approvals, the period of time during which we could market any of our product candidates under patent protection, if approved, would be reduced. Since patent applications in the United States and most other countries are confidential for a period of time after filing, and some remain so until issued, we cannot be certain that we were the first to file any patent application related to our product candidates. Furthermore, an interference proceeding can be provoked by a third party or instituted by the U.S. Patent and Trademark Office, or USPTO, to determine who was the first to invent any of the subject matter covered by the patent claims of our applications. In addition, patents have a limited lifespan. In the United States, the natural expiration of a patent is generally 20 years after it is filed. Various extensions may be available; however, the life of a patent, and the protection it affords, is limited. Even if we obtain patents that cover the manufacture, use and/or sale of

our product candidates and such patents are not successfully challenged by any third parties, once the patent life has expired for a product, we may be open to competition, including from generic medications.

We may not be successful in obtaining or maintaining necessary rights to product components and processes for our development pipeline through acquisitions and in-licenses.

Presently we have rights to certain intellectual property through licenses from third parties and under patents that we own or co-own, related to a subset of the known microRNA targets. Because our programs may involve a range of microRNA targets and specific formulations of microRNA mimics directed to such targets, including targets and formulations that may require the use of proprietary rights held by third parties, the growth of our business will likely depend in part on our ability to acquire, in-license or otherwise gain the right to use these proprietary rights. We may be unable to acquire or in-license any necessary or desirable third-party intellectual property rights on reasonable terms, or at all. The licensing and acquisition of third-party intellectual property rights is a competitive area, and a number of more established companies are also pursuing strategies to license or acquire third-party intellectual property rights that we may consider attractive now or in the future. These established companies may have a competitive advantage over us due to their size, cash resources and greater clinical development and commercialization capabilities. In addition, companies that perceive us to be a competitor may be unwilling to assign or license rights to us. We also may be unable to license or acquire third-party intellectual property rights on terms that would allow us to make an appropriate return on our investment. If we are unable to successfully obtain rights to required third-party intellectual property rights, including rights related to our lead product candidate, our business, financial condition and prospects for growth could suffer.

If we are unable to protect the confidentiality of our trade secrets, the value of our technology could be materially adversely affected and our business would be harmed.

In addition to the protection afforded by patents, we rely on trade secret protection and confidentiality agreements to protect proprietary know-how that may not be patentable or that we elect not to patent, processes for which patents may be difficult to obtain or enforce, and any other elements of our product candidates' discovery and development processes that involve proprietary know-how, information or technology that is not covered by patents. However, trade secrets can be difficult to protect. We seek to protect our proprietary data and processes, in part, by confidentiality agreements and invention assignment agreements with our employees, consultants, scientific advisors, contractors and collaborators, and any other third parties that have access to our proprietary know-how, information or technology, for example, third parties involved in our clinical trials. Although we expect all of our employees, consultants, advisors and any third parties who have access to our proprietary know-how, information or technology to enter into confidentiality agreements, we cannot provide any assurances that all such agreements have been duly executed, and we cannot be certain that our trade secrets and other confidential proprietary information will not be disclosed or that competitors will not otherwise gain access to our trade secrets or independently develop substantially equivalent information and techniques. Misappropriation or unauthorized disclosure of our trade secrets could impair our competitive position and may have a material adverse effect on our business. Additionally, if the steps taken to maintain our trade secrets are deemed inadequate, we may have insufficient recourse against third parties for misappropriating the trade secret. In addition, others may independently discover our trade secrets and proprietary information. For example, the FDA, as part of its Transparency Initiative, is currently considering whether to make additional information publicly available on a routine basis, including information that we may consider to be trade secrets or other proprietary information, and it is not clear at the present time how the FDA's disclosure policies may change in the future, if at all.

We also seek to preserve the integrity and confidentiality of our data, trade secrets and know-how by maintaining physical security of our premises and physical and electronic security of our information technology systems. While we have confidence in these individuals, organizations and systems, agreements or security measures may be breached, and we may not have adequate remedies for any breach. Moreover, if any of our trade secrets were to be lawfully obtained or independently developed by a competitor, we would have no right to prevent such competitor from using that technology or information to compete with us, which could harm our competitive position.

Further, the laws of some foreign countries do not protect proprietary rights to the same extent or in the same manner as the laws of the United States. As a result, we may encounter significant problems in protecting and defending our intellectual property both in the United States and abroad. If we are unable to prevent material disclosure of the

intellectual property related to our technologies to third parties, we will not be able to establish or maintain a competitive advantage in our market, which could materially adversely affect our business, financial condition and results of operations.

Issued patents covering our product candidates could be found invalid or unenforceable if challenged in court or the USPTO.

If we or one of our licensing partners initiated legal proceedings against a third party to enforce a patent covering the manufacture, use or sale, or other aspects of one of our product candidates, the defendant could counterclaim that the patent covering our product candidate is invalid and/or unenforceable. In patent litigation in the United States, defendant counterclaims alleging invalidity and/or unenforceability are commonplace, and there are numerous grounds upon which a third party can assert invalidity or unenforceability of a patent. Third parties may also raise similar claims before administrative bodies in the United States or abroad, even outside the context of litigation. Such mechanisms include ex parte re-examination, inter partes review, post grant review and equivalent proceedings in foreign jurisdictions (e.g., opposition proceedings). Such proceedings could result in revocation or amendment to our patents in such a way that they no longer cover our product candidates. The outcome following legal assertions of invalidity and unenforceability is unpredictable. With respect to the validity question, for example, we cannot be certain that there is no invalidating prior art, of which we, our patent counsel and the patent examiner were unaware during prosecution. If a defendant were to prevail on a legal assertion of invalidity and/or unenforceability, we would lose at least part, and perhaps all, of the patent protection on our product candidates. Similarly, the outcome following administrative review of a patent that we own or license, such as via a reexamination or opposition proceeding before the USPTO or a foreign body, is unpredictable. If a third party were to prevail, we could lose at least part, and perhaps all, of the patent protection on our product candidates. Such a loss of patent protection could have a material adverse impact on our business.

If we are sued for infringing the patent rights or misappropriating the trade secrets of third parties, such litigation could be costly and time consuming and could prevent or delay us from developing or commercializing our product candidates.

Our commercial success depends, in part, on our ability to develop, manufacture, market and sell our product candidates and use our proprietary technology without infringing the patent rights of third parties. Numerous third-party U.S. and non-U.S. issued patents and pending applications exist in the area of microRNA. We are aware of certain U.S. and foreign patents and pending patent applications owned by our competitors or other third parties that cover certain miR-34 mimics and therapeutic uses thereof. We are currently monitoring these patents and patent applications. We have and we may in the future pursue available proceedings in the U.S. and foreign patent offices to challenge the validity of these patents and patent applications. In addition, or alternatively, we may consider whether to seek to negotiate a license of rights to technology covered by one or more of such patents and patent applications. If any patents or patent applications cover our product candidates or technologies, we may not be free to manufacture or market our product candidates, including MRX34, as planned, absent such a license, which may not be available to us on commercially reasonable terms, or at all.

It is also possible that we have failed to identify relevant third-party patents or applications. For example, applications filed before November 29, 2000 and certain applications filed after that date that will not be filed outside the United States remain confidential until patents issue. Moreover, it is difficult for industry participants, including us, to identify all third-party patent rights that may be relevant to our product candidates and technologies because patent searching is imperfect due to differences in terminology among patents, incomplete databases and the difficulty in assessing the meaning of patent claims. We may fail to identify relevant patents or patent applications or may identify pending patent applications of potential interest but incorrectly predict the likelihood that such patent applications may issue with claims of relevance to our technology. In addition, we may be unaware of one or more issued patents that would be infringed by the manufacture, sale or use of a current or future product candidate, or we may incorrectly conclude that a third-party patent is invalid, unenforceable or not infringed by our activities. Additionally, pending patent applications that have been published can, subject to certain limitations, be later amended in a manner that could cover our technologies, our products or the use of our products.

There is a substantial amount of intellectual property litigation in the biotechnology and pharmaceutical industries, and we may become party to, or threatened with, litigation or other adversarial proceedings regarding patent rights with respect to our technology or products candidates, including interferences, oppositions and *inter partes* review proceedings before the USPTO and corresponding foreign patent offices. We also monitor patent prosecution activities and pending applications of competitors and potential competitors in our field in order to identify third party patent rights that could pose a potential threat to our freedom to operate in the market with respect to our product candidates, once commercialized. We are currently pursuing and may in the future pursue available administrative proceedings in the U.S. or foreign patent offices to challenge third party patent rights that could adversely impact our ability to commercialize one or more of our product candidates. As the biotechnology and pharmaceutical industries expand and more patents are issued, the risk increases that our current or future product candidates may be subject to claims of infringement of the patent rights of third parties, who may assert infringement claims against us based on existing or future patent rights. Third parties may assert that we are employing their proprietary technology without authorization. There may be third-party patents or patent applications with claims to materials, formulations, methods of manufacture or methods for treatment related to the use or manufacture of our product candidates and third parties could allege that our technology infringes such claims. Further, because patent applications can take many years to issue, third parties may have currently pending patent applications which may later result in issued patents that our product candidates may infringe, or which such third parties claim are infringed by the use of our technologies. The outcome of patent litigation is subject to uncertainties that cannot be adequately quantified in advance. The pharmaceutical and biotechnology industries have produced a significant number of patents, and it may not always be clear to industry participants, including us, which patents cover various types of products or methods of use. The coverage of patents is subject to interpretation by the courts, and the interpretation is not always uniform. If we are sued for patent infringement, we would need to demonstrate that our product candidates, products or methods either do not infringe the patent claims of the relevant patent or that the patent claims are invalid, and we may not be able to do this. Proving that a patent is invalid is difficult. For example, in the United States, proving invalidity requires a showing of clear and convincing evidence to overcome the presumption of validity enjoyed by issued patents. Even if we are successful in these proceedings, we may incur substantial costs and the time and attention of our management and scientific personnel could be diverted in pursuing these proceedings, which could have a material adverse effect on us. In addition, we may not have sufficient resources to bring these actions to a successful conclusion.

If we are found to infringe a third party's patent rights, including any patent rights related to miR-34, we could be forced, including by court order, to cease developing, manufacturing or commercializing the infringing product candidate or product. Alternatively, we may be required to obtain a license from such third party in order to use the infringing technology and continue developing, manufacturing or marketing the infringing product candidate. However, we may not be able to obtain any required license on commercially reasonable terms or at all. Even if we were able to obtain a license, it could be non-exclusive, thereby giving our competitors access to the same technologies licensed to us. In addition, we could be found liable for monetary damages, including treble damages and attorneys' fees if we are found to have willfully infringed a patent. A finding of infringement could prevent us from commercializing our product candidates or force us to cease some of our business operations, which could materially harm our business. Claims that we have misappropriated the confidential information or trade secrets of third parties could have a similar negative impact on our business.

Parties making claims against us for infringement of their patent rights may obtain injunctive or other equitable relief, which could effectively block our ability to further develop and commercialize one or more of our product candidates. Defense of these claims, regardless of their merit, would involve substantial litigation expense and would be a substantial diversion of employee resources from our business. In the event of a successful claim of infringement against us, we could be required to redesign our infringing products or obtain a license from such third party to continue developing and commercializing our products and technology. However, we may not be able to obtain any required license on commercially reasonable terms, or at all. Even if we are able to obtain a license, it may be non-exclusive, thereby giving our competitors access to the same technologies licensed to us. It may be impossible to redesign our products and technology, or it may require substantial time and monetary expenditure, which could force us to cease commercialization of one or more of our product candidates, including MRX34, or some of our business operations, which could materially harm our business. In addition, in any such proceeding, we may be required to pay substantial damages, including treble damages and attorneys' fees in the event we are found liable for willful infringement.

We may be involved in lawsuits or administrative proceedings to protect or enforce our intellectual property rights, which could be expensive, time consuming and unsuccessful.

Competitors may infringe or we may believe that they infringe patents that we own or license. To counter infringement or unauthorized use, we may be required to file infringement claims, which can be expensive and time-consuming. In addition, in an infringement proceeding, a court may decide that a patent of ours is not valid or is unenforceable, or may refuse to stop the other party in such infringement proceeding from using the technology at issue on the grounds that our patents do not cover the technology in question. An adverse result in any litigation or defense proceedings could put one or more of our patents at risk of being invalidated, held unenforceable or interpreted narrowly, and could put any of our patent applications at risk of not yielding an issued patent. Litigation is uncertain, and we cannot predict whether we would be successful in any such litigation.

Interference proceedings provoked by third parties or brought by the USPTO or any foreign patent authority may be necessary to determine the priority of inventions with respect to our patents or patent applications. An unfavorable outcome could require us to cease using the related technology or to attempt to license rights to it from the prevailing party. Our business could be harmed if the prevailing party does not offer us a license on commercially reasonable terms, if any license is offered at all. Litigation or interference proceedings may fail and, even if successful, may result in substantial costs and distract our management and other employees.

We may not be able to prevent misappropriation of our trade secrets or confidential information, particularly in countries where the laws may not protect those rights as fully as in the United States. Furthermore, because of the substantial amount of discovery required in connection with intellectual property litigation, there is a risk that some of our confidential information could be compromised by disclosure during this type of litigation. In addition, there could be public announcements of the results of hearings, motions or other interim proceedings or developments. If securities analysts or investors perceive these results to be negative, it could have a substantial adverse effect on the price of our common stock.

Legal actions to enforce patent rights or other intellectual property rights that we own or license can be expensive and may involve the diversion of significant management time. In addition, these legal actions could be unsuccessful and could also result in the invalidation of our patents or a finding that they are unenforceable. Moreover, third parties may be able to successfully design around our patents using pre-existing technology, by developing new technology or by using similar technology that is outside the scope of our patents. We may or may not choose to pursue litigation against those that have infringed on our patents, or used them without authorization, due to the associated expense and time commitment of monitoring these activities. If we fail to protect or to enforce our intellectual property rights successfully, our competitive position could suffer, which could harm our results of operations.

We may not be able to protect our intellectual property rights throughout the world.

Filing, prosecuting and defending patents on all of our product candidates throughout the world would be prohibitively expensive, and our patent rights in some countries outside the United States can be less extensive than those in the United States. The requirements for patentability may differ in certain countries, particularly developing countries. For example, unlike other countries, China has a heightened requirement for patentability, and specifically requires a detailed description of medical uses of a claimed therapeutic. In addition, the laws of some foreign countries do not protect intellectual property rights to the same extent as federal and state laws in the United States. Consequently, we may not be able to prevent third parties from practicing our inventions in all countries outside the United States, or from selling or importing products made using our inventions in and into the United States or other jurisdictions.

As part of ordinary course prosecution and maintenance activities, we determine whether to seek patent protection outside the United States and in which countries. This also applies to patents we have acquired or in-licensed from third parties. In some cases, this means that we, or our predecessors in interest or licensors of patents within our portfolio, have sought patent protection in a limited number of countries for patents covering our product candidates, including for patents providing coverage for MRX34. Competitors may use our technologies in jurisdictions where we have not pursued and obtained patent protection to develop their own products and, further, may export otherwise infringing products to territories where we have patent protection but where enforcement is not as strong as in the United

States. These products may compete with our products in jurisdictions where we do not have any issued patents and, even in jurisdictions where we have or are able to obtain issued patents, our patent claims or other intellectual property rights may not be effective or sufficient to prevent them from so competing. Many companies have encountered significant problems in protecting and defending intellectual property rights in foreign jurisdictions. The legal systems of certain countries, particularly certain developing countries, do not favor the enforcement of patents and other intellectual property protection, particularly those relating to biopharmaceuticals, which could make it difficult for us to stop the infringement of our patents or marketing of competing products in violation of our proprietary rights generally. Proceedings to enforce our patent rights in foreign jurisdictions could result in substantial cost and divert our efforts and attention from other aspects of our business, could put our patents at risk of being invalidated or interpreted narrowly and our patent applications at risk of not issuing and could provoke third parties to assert claims against us. We may not prevail in any lawsuits that we initiate and the damages or other remedies awarded, if any, may not be commercially meaningful. In addition, certain countries in Europe and certain developing countries, including India and China, have compulsory licensing laws under which a patent owner may be compelled to grant licenses to third parties. In those countries, we may have limited remedies if our patents are infringed or if we are compelled to grant a license to our patents to a third party, which could materially diminish the value of those patents. This could limit our potential revenue opportunities.

Accordingly, our efforts to enforce our intellectual property rights around the world may be inadequate to obtain a significant commercial advantage from the intellectual property that we own or license. Moreover, patent protection must ultimately be sought on a country-by-country basis, which is an expensive and time-consuming process with uncertain outcomes. Accordingly, we may choose not to seek patent protection in certain countries, and we will not have the benefit of patent protection in such countries.

The patent protection and patent prosecution for some of our product candidates may be dependent on our third party licensors.

While we normally seek to obtain the right to control the filing, prosecution, maintenance, defense and enforcement of the patents and patent applications that we in-license relating to our product candidates, there may be times when such activities for patents that relate to our product candidates are controlled by our licensors. For example, we do not have the first right to prosecute, maintain, defend, or enforce the patent rights licensed to us relating to the SMARTICLES technology under our agreement with Marina Biotech, Inc., or Marina. Although we may retain the right to consult on such filing, prosecution, maintenance, defense, and enforcement activities, our overall ability to influence such activities is limited. Moreover, the patent rights we have in-licensed from Marina may be put at risk in litigation or administrative proceedings unrelated to our product candidates. Further, while we seek to have rights to take action to defend our in-licensed patents and patent applications from third-party challenges in the event that our licensors determine not to, we may not be aware of any such potential threats to the intellectual property rights we in-license, or we may be unsuccessful in protecting such intellectual property rights if we respond to any such challenges by third parties.

If these licensors or any of our future licensors fail to appropriately file, prosecute, maintain, defend or enforce our in-licensed patents and patent applications covering any of our product candidates, our ability to develop and commercialize those product candidates may be adversely affected and we may not be able to prevent competitors from making, using and selling competing products.

If we breach any of the agreements under which we license patent rights to use, develop and commercialize our product candidates or our technologies from third parties or, in certain cases, we fail to meet certain development deadlines, we could lose license rights that are important to our business.

We are a party to a number of license agreements under which we are granted rights to intellectual property that are important to our business and we expect that we may need to enter into additional license agreements in the future. These include our exclusive cross-license agreement with Asuragen, our exclusive licenses from Yale University, or Yale, Marina, the University of Zurich, and Rosetta Genomics.

Our existing license agreements, except our cross-license agreement with Asuragen, generally impose, and we expect that future license agreements will impose on us, various development, regulatory and/or commercial diligence obligations, and financial obligations, such as payment of milestones and/or royalties. If we fail to comply with our obligations under these agreements, or we are subject to a bankruptcy, the licensor may have the right to terminate the license, in which event we may not be able to market products covered by the license. Our business could suffer, for example, if any current or future licenses terminate, if the licensors fail to abide by the terms of the license, if the licensed patents or other rights are found to be invalid or unenforceable, or if we are unable to enter into necessary licenses on acceptable terms. See "Business—Key Partnerships and Licenses" for a description of our license agreements, which sets forth the material terms and obligations, including a description of the termination provisions, under our agreements with Asuragen, Yale, Marina, the University of Zurich and Rosetta Genomics.

We license the technology related to SMARTICLES from Marina. Our license with Marina imposes various development, regulatory, commercial diligence, financial and other obligations. If we fail to comply with our obligations under the agreement with Marina, or otherwise materially breach the agreement with Marina, and fail to remedy such failure or cure such breach, Marina may have the right to terminate the license. The loss of the license from Marina would affect a portion of the patent portfolio for MRX34, which would adversely affect our ability to proceed with any development or potential commercialization of MRX34, and could subject us to claims of patent infringement by Marina if MRX34 is covered by the affected patents.

As we have done previously, we may need to obtain licenses from third parties to advance our research or allow commercialization of our product candidates, and we cannot provide any assurances that third-party patents do not exist that might be enforced against our current product candidates or future products in the absence of such a license. We may fail to obtain any of these licenses on commercially reasonable terms, if at all. Even if we are able to obtain a license, it may be non-exclusive, thereby giving our competitors access to the same technologies licensed to us. In that event, we may be required to expend significant time and resources to develop or license replacement technology. If we are unable to do so, we may be unable to develop or commercialize the affected product candidates, which could materially harm our business and the third parties owning such intellectual property rights could seek either an injunction prohibiting our sales, or, with respect to our sales, an obligation on our part to pay royalties and/or other forms of compensation.

Licensing of intellectual property is of critical importance to our business and involves complex legal, business and scientific issues. Disputes may arise between us and our licensors regarding intellectual property subject to a license agreement, including:

- the scope of rights granted under the license agreement and other interpretation-related issues;
- whether and the extent to which our technology and processes infringe on intellectual property of the licensor that is not subject to the licensing agreement;
- our right to sublicense patent and other rights to third parties under collaborative development relationships;
- our diligence obligations with respect to the use of the licensed technology in relation to our development and commercialization of our product candidates, and what activities satisfy those diligence obligations; and
- the ownership of inventions and know-how resulting from the joint creation or use of intellectual property by our licensors and us and our partners.

If disputes over intellectual property that we have licensed arise, we would expect to exercise all rights and remedies available to us, including seeking to cure any breach by us, and otherwise seek to preserve our rights under the patents licensed to us. However, we may not be able to do so in a timely manner, at an acceptable cost or at all. Generally, the loss of any one of our current licenses, or any other license we may acquire in the future, could prevent or

impair our ability to successfully develop and commercialize the affected product candidates and thus materially harm our business, prospects, financial condition and results of operations.

Intellectual property rights do not necessarily address all potential threats to our competitive advantage.

The degree of future protection afforded by our intellectual property rights is uncertain because intellectual property rights have limitations, and may not adequately protect our business, provide a barrier to entry against our competitors or potential competitors, or permit us to maintain our competitive advantage. Moreover, if a third party has intellectual property rights that cover the practice of our technology, we may not be able to fully exercise or extract value from our intellectual property rights. The following examples are illustrative:

- others may be able to make compounds that are similar to our product candidates but that are not covered by the claims of the patents that we own or license;
- we or our licensors or collaborators might not have been the first to make the inventions covered by an issued patent or pending patent application that we own or license;
- we or our licensors or collaborators might not have been the first to file patent applications covering an invention;
- others may independently develop similar or alternative technologies or duplicate any of our technologies without infringing or misappropriating our intellectual property rights;
- pending patent applications that we own or license may not lead to issued patents;
- issued patents that we own or license may not provide us with any competitive advantages, or may be held invalid or unenforceable, as a result of legal challenges by our competitors;
- our competitors might conduct research and development activities in countries where we do not have patent rights and then use the information learned from such activities to develop competitive products for sale in our major commercial markets;
- we may not develop or in-license additional proprietary technologies that are patentable; and
- the patents of others may have an adverse effect on our business.

Should any of these events occur, they could significantly harm our business, results of operations and prospects.

We may be subject to claims challenging the inventorship or ownership of our patents and other intellectual property.

We were previously involved in discussions with Yale regarding the inventorship and ownership of certain patents and patent applications licensed to us by Asuragen. An independent third party expert was engaged to determine the inventorship and the ownership of patents and patent applications potentially subject to Yale and Asuragen co-ownership. This determination confirmed Asuragen’s sole ownership of the patents and patent applications where co-ownership had been under consideration and resulted in a determination that Yale should be removed as a co-owner of one of the pending patent applications, an action we are currently undertaking.

Although we seek to protect our ownership of our patents and other intellectual property by ensuring that our agreements with our employees and certain collaborators and other third parties with whom we do business include provisions requiring, for instance, such parties to assign rights in inventions to us, we may be subject to claims that former or current employees, collaborators or other third parties have an ownership interest in our patents, in-licensed patents or other intellectual property. In some situations, our confidentiality agreements may conflict with, or be subject to, the rights of third parties with whom our employees, consultants or advisors have previous employment or consulting

relationships, and further, many of our consultants are currently retained by other biotechnology or pharmaceutical companies, including our competitors or potential competitors, and may be subject to conflicting obligations to these third parties. To the extent that our employees, consultants or contractors use any intellectual property owned by third parties in their work for us, disputes may arise as to the ownership of rights in any related or resulting know-how and inventions, arising, for example, from such conflicting obligations of consultants, employees or others who are involved in developing our product candidates. Litigation may be necessary to defend against these and other claims challenging inventorship or ownership. If we fail in defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights, such as exclusive ownership of, or right to use, valuable intellectual property. Such an outcome could have a material adverse effect on our business. Even if we are successful in defending against such claims, litigation could result in substantial costs and be a distraction to management and other employees.

Obtaining and maintaining patent protection depends on compliance with various procedural, document submission, fee payment and other requirements imposed by governmental patent agencies, and our patent protection could be reduced or eliminated for non-compliance with these requirements.

The USPTO and various foreign governmental patent agencies require compliance with a number of procedural, documentary, fee payment and other similar provisions during the patent prosecution process. Periodic maintenance fees, renewal fees, annuity fees and various other governmental fees on patents and/or patent applications will be due to be paid to the USPTO and various patent agencies outside of the United States in several stages over the lifetime of the patents and/or applications. We have systems in place to remind us to pay these fees, and we employ reputable law firms and other professionals and rely on such third parties to effect payment of these fees with respect to the USPTO and non-U.S. patent agencies with respect to the patents and patent applications we own, and we rely upon our licensors to effect payment of these fees with respect to the patents and patent applications that we in-license. Even if we do not control prosecution and maintenance of our in-licensed patents, we may be responsible for reimbursing our licensors for some or all of the costs associated with such activities. If we fail to make timely payment to our licensors for such fees, our licensors may have the right to terminate the affected license, in which event we would not be able to market products covered by the license. We also employ reputable law firms and other professionals to help us comply with the various documentary and other procedural requirements with respect to the patents and patent applications that we own. In some cases, an inadvertent lapse can be cured by payment of a late fee or by other means in accordance with the applicable rules. However, there are situations in which noncompliance can result in abandonment or lapse of the patent or patent application, resulting in partial or complete loss of patent rights in the relevant jurisdiction. In such an event, our competitors might be able to enter the market and this circumstance would have a material adverse effect on our business.

Changes in U.S. patent law could diminish the value of patents in general, thereby impairing our ability to protect our products, and recent patent reform legislation could increase the uncertainties and costs surrounding the prosecution of our patent applications and the enforcement or defense of our issued patents.

As is the case with other biotechnology companies, our success is heavily dependent on patents. Obtaining and enforcing patents in the biotechnology industry involve both technological and legal complexity, and is therefore costly, time-consuming and inherently uncertain. In addition, the United States has recently enacted and is currently implementing wide-ranging patent reform legislation. Recent U.S. Supreme Court rulings have narrowed the scope of patent protection available in certain circumstances and weakened the rights of patent owners in certain situations. In addition to increasing uncertainty with regard to our ability to obtain patents in the future, this combination of events has created uncertainty with respect to the value of patents, once obtained. Depending on decisions by the U.S. Congress, the federal courts, and the USPTO, the laws and regulations governing patents could change in unpredictable ways that would weaken our ability to obtain new patents or to enforce our existing patents and patents that we might obtain in the future. Some of our patent claims may be affected by the recent U.S. Supreme Court decision in *Association for Molecular Pathology v. Myriad Genetics*. In *Myriad*, the Supreme Court held that unmodified isolated fragments of genomic sequences, such as the DNA constituting the BRCA1 and BRCA2 genes, are not eligible for patent protection because they constitute a product of nature. The exact boundaries of the Supreme Court’s decision remain unclear as the Supreme Court did not address other types of nucleic acids, such as isolated microRNAs. Nevertheless, our patent portfolio contains claims of various types and scope, including chemically modified mimics, such as in MRX34, as well as methods of medical treatment. In our view, the presence of varying claims in our patent portfolio significantly

reduces, but does not eliminate, our exposure to potential validity challenges under *Myriad* or future judicial decisions. However, it is not yet clear what, if any, impact this recent Supreme Court decision or future decisions will have on the operation of our business.

For our U.S. patent applications containing a claim not entitled to priority before March 16, 2013, there is a greater level of uncertainty in the patent law. On September 16, 2011, the Leahy-Smith America Invents Act, or the Leahy-Smith Act, was signed into law. The Leahy-Smith Act includes a number of significant changes to U.S. patent law. These include provisions that affect the way patent applications will be prosecuted and may also affect patent litigation. The USPTO has promulgated regulations and developed procedures to govern administration of the Leahy-Smith Act, and many of the substantive changes to patent law associated with the Leahy-Smith Act, and in particular, the first to file provisions, did not come into effect until March 16, 2013. Accordingly, it is not yet clear what, if any, impact the Leahy-Smith Act will have on the operation of our business. However, the Leahy-Smith Act and its implementation could increase the uncertainties and costs surrounding the prosecution of our patent applications and the enforcement or defense of our issued patents, all of which could have a material adverse effect on our business and financial condition.

An important change introduced by the Leahy-Smith Act is that, as of March 16, 2013, the United States transitioned to a "first-to-file" system for deciding which party should be granted a patent when two or more patent applications are filed by different parties claiming the same invention. A third party that files a patent application in the USPTO after that date but before us could therefore be awarded a patent covering an invention of ours even if we had made the invention before it was made by the third party. This will require us to be cognizant going forward of the time from invention to filing of a patent application. Furthermore, our ability to obtain and maintain valid and enforceable patents depends on whether the differences between our technology and the prior art allow our technology to be patentable over the prior art. Since patent applications in the United States and most other countries are confidential for a period of time after filing, we cannot be certain that we were the first to either (i) file any patent application related to our product candidates or (ii) invent any of the inventions claimed in our patents or patent applications.

Among some of the other changes introduced by the Leahy-Smith Act are changes that limit where a patentee may file a patent infringement suit and providing opportunities for third parties to challenge any issued patent in the USPTO. This applies to all of our U.S. patents, even those issued before March 16, 2013. Because of a lower evidentiary standard in USPTO proceedings compared to the evidentiary standard in United States federal court necessary to invalidate a patent claim, a third party could potentially provide evidence in a USPTO proceeding sufficient for the USPTO to hold a claim invalid even though the same evidence would be insufficient to invalidate the claim if first presented in a district court action. Accordingly, a third party may attempt to use the USPTO procedures to invalidate our patent claims that would not have been invalidated if first challenged by the third party as a defendant in a district court action.

We may be subject to claims that our employees or consultants or independent contractors have wrongfully used or disclosed confidential information or trade secrets of third parties or that our employees or consultants have wrongfully used or disclosed alleged trade secrets of former or other employers.

Many of our employees, independent contractors and consultants, including our senior management, have been previously employed or retained by other biotechnology or pharmaceutical companies, including our competitors or potential competitors. Although we try to ensure that our employees, consultants and independent contractors do not use the proprietary information or know-how of third parties in their work for us, and do not perform work for us that is in conflict with their obligations to another employer or any other entity, we may be subject to claims that we or our employees, consultants or independent contractors have inadvertently or otherwise improperly used or disclosed confidential information, including trade secrets or other proprietary information, of a former employer or other third parties. We may also be subject to claims that an employee, advisor, consultant, or independent contractor performed work for us that conflicts with that person's obligations to a third party, such as an employer, and thus, that the third party has an ownership interest in the intellectual property arising out of work performed for us. We are not aware of any threatened or pending claims related to these matters, but in the future litigation may be necessary to defend against such claims. If we fail in defending any such claims, in addition to paying monetary damages, we may lose valuable personnel or intellectual property rights, such as exclusive ownership of, or right to use, valuable intellectual property. Such an

outcome could have a material adverse effect on our business. Even if we are successful in defending against such claims, litigation could result in substantial costs and be a distraction to management.

Intellectual property disputes could cause us to spend substantial resources and distract our personnel from their normal responsibilities.

Even if resolved in our favor, litigation or other legal proceedings relating to intellectual property claims may cause us to incur significant expenses, and could distract our technical and/or management personnel from their normal responsibilities. In addition, there could be public announcements of the results of hearings, motions or other interim proceedings or developments and if securities analysts or investors perceive these results to be negative, it could have a substantial adverse effect on the market price of our common stock. Such litigation or proceedings could substantially increase our operating losses and reduce the resources available for development activities or any future sales, marketing or distribution activities. We may not have sufficient financial or other resources to adequately conduct such litigation or proceedings. Some of our competitors may be able to sustain the costs of such litigation or proceedings more effectively than we can because of their greater financial resources. Uncertainties resulting from the initiation and continuation of patent litigation or other proceedings could have a material adverse effect on our ability to compete in the marketplace.

If we do not obtain patent term extension in the United States under the Hatch-Waxman Act and in foreign countries under similar legislation, thereby potentially extending the term of our marketing exclusivity for our product candidates, our business may be materially harmed.

Depending upon the timing, duration and specifics of FDA marketing approval of our product candidates, if any, one of the U.S. patents covering each of such approved product(s) or the use thereof may be eligible for up to five years of patent term restoration under the Hatch-Waxman Act. The Hatch-Waxman Act allows a maximum of one patent to be extended per FDA approved product. Patent term extension also may be available in certain foreign countries upon regulatory approval of our product candidates. Nevertheless, we may not be granted patent term extension either in the United States or in any foreign country because of, for example, failing to apply within applicable deadlines, failing to apply prior to expiration of relevant patents or otherwise failing to satisfy applicable requirements. Moreover, the term of extension, as well as the scope of patent protection during any such extension, afforded by the governmental authority could be less than we request. In addition, if a patent we wish to extend is owned by another party and licensed to us, we may need to obtain approval and cooperation from our licensor to request the extension.

If we are unable to obtain patent term extension or restoration, or the term of any such extension is less than we request, the period during which we will have the right to exclusively market our product will be shortened and our competitors may obtain approval of competing products following our patent expiration, and our revenue could be reduced, possibly materially.

Risks Related to Government Regulation

Even if we receive regulatory approval for a product candidate, we will be subject to ongoing regulatory obligations and continued regulatory review, which may result in significant additional expense and subject us to penalties if we fail to comply with applicable regulatory requirements.

Once regulatory approval has been granted, the approved product and its manufacturer are subject to continual review by the FDA and/or non-U.S. regulatory authorities. Any regulatory approval that we receive for our product candidates may be subject to limitations on the indicated uses for which the product may be marketed or contain requirements for potentially costly post- marketing follow-up studies to monitor the safety and efficacy of the product. In addition, if the FDA and/or non-U.S. regulatory authorities approve any of our product candidates, we will be subject to extensive and ongoing regulatory requirements by the FDA and other regulatory authorities with regard to the labeling, packaging, adverse event reporting, storage, sampling, advertising, promotion and recordkeeping for our products. Manufacturers of our products are required to comply with cGMP regulations, which include requirements related to quality control and quality assurance as well as the corresponding maintenance of records and documentation. Further, regulatory authorities must approve these manufacturing facilities before they can be used to manufacture our products, and these facilities are subject to continual review and periodic inspections by the FDA and other regulatory authorities

for compliance with cGMP regulations. Accordingly, we and others with whom we work must continue to expend time, money, and effort in all areas of regulatory compliance, including manufacturing, production, and quality control. We will also be required to report certain adverse reactions and production problems, if any, to the FDA, and to comply with requirements concerning advertising and promotion for our products. Promotional communications with respect to prescription drugs are subject to a variety of legal and regulatory restrictions and must be consistent with the information in the product's approved label. As such, we may not promote our products for indications or uses for which they do not have FDA approval.

If we, any current or future collaborator or a regulatory authority discovers previously unknown problems with a product, such as adverse events of unanticipated severity or frequency, or problems with the facility where the product is manufactured, a regulatory authority may impose restrictions on that product, such collaborator, the manufacturer or us, including requiring withdrawal of the product from the market or suspension of manufacturing. If we, our product candidates or the manufacturing facilities for our product candidates fail to comply with regulatory requirements of the FDA and/or other non-U.S. regulatory authorities, we could be subject to administrative or judicially imposed sanctions, including:

- warning letters;
- civil or criminal penalties;
- injunctions;
- suspension of or withdrawal of regulatory approval;
- total or partial suspension of any ongoing clinical trials or of production;
- voluntary or mandatory product recalls and publicity requirements;
- refusal to approve pending applications for marketing approval of new products or supplements to approved applications filed by us;
- restrictions on operations, including costly new manufacturing requirements; or
- seizure or detention of our products or import bans.

The regulatory requirements and policies may change and additional government regulations may be enacted for which we may also be required to comply. We cannot predict the likelihood, nature or extent of government regulation that may arise from future legislation or administrative action, either in the United States or in other countries. Any new regulations or revisions or reinterpretations of existing regulations may impose additional costs or lengthen review times of our product candidates. In addition, if we or any current or future collaborator are not able to maintain regulatory compliance, we or such collaborator, as applicable, will not be permitted to market our future products and our business will suffer.

The availability of adequate third-party coverage and reimbursement for newly approved products is uncertain, and failure to obtain adequate coverage and reimbursement from third-party payors could impede our ability to market any future products we may develop and could limit our ability to generate revenue.

There is significant uncertainty related to the third-party payor coverage and reimbursement of newly approved medical products. The commercial success of our future products in both domestic and international markets depends on whether such third-party coverage and reimbursement are available for our future products. Governmental payors, including Medicare and Medicaid, health maintenance organizations and other third-party payors are increasingly attempting to manage their healthcare expenditures and challenging the prices charged for medical products and services by limiting both coverage and the level of reimbursement of new drugs and biologics and, as a result, they may not cover or provide adequate reimbursement for our future products. These payors may not view our future products as

cost-effective, and coverage and reimbursement may not be available to our customers, may be limited to certain indications or may not be sufficient to allow our future products to be marketed on a competitive basis. Third-party payors are exerting increasing influence on decisions regarding the use of, and coverage and reimbursement levels for, particular treatments. Cost-control initiatives could cause us to decrease the price we might establish for our products candidates, which could result in lower than anticipated product revenues. If we decrease the prices for our product candidates because of competitive pressures or if governmental and other third-party payors do not provide adequate coverage or reimbursement, our prospects for revenue and profitability will suffer.

If we fail to comply or are found to have failed to comply with FDA and other regulations related to the promotion of our products for unapproved uses, we could be subject to criminal penalties, substantial fines or other sanctions and damage awards.

The regulations relating to the promotion of products for unapproved uses are complex and subject to substantial interpretation by the FDA and other government agencies. If we receive marketing approval for MRX34 or other product candidates, we will be restricted from promoting the products for uses outside of the approved labeling. However, physicians may nevertheless prescribe products to their patients in a manner that is inconsistent with the approved label. We intend to implement compliance and training programs designed to ensure that our sales and marketing practices comply with applicable regulations. Notwithstanding these programs, the FDA or other government agencies may allege or find that our practices constitute prohibited promotion of our products for unapproved uses. We also cannot be sure that our employees will comply with company policies and applicable regulations regarding the promotion of products for unapproved uses.

Over the past several years, a significant number of pharmaceutical and biotechnology companies have been the target of inquiries and investigations by various federal and state regulatory, investigative, prosecutorial and administrative entities in connection with the promotion of products for unapproved uses and other sales practices, including the Department of Justice and various U.S. Attorneys' Offices, the Office of Inspector General of the Department of Health and Human Services, the FDA, the Federal Trade Commission and various state Attorneys General offices. These investigations have included claims asserting alleged violations of various federal and state laws and regulations, including antitrust laws, the Food, Drug and Cosmetic Act, the False Claims Act, the Prescription Drug Marketing Act, anti-kickback laws, and other alleged violations in connection with the promotion of products for unapproved uses, pricing and reimbursement from government programs such as the Medicare and Medicaid programs. Many of these investigations originate as "qui tam" actions, commonly referred to as "whistleblower suits," under the False Claims Act, often brought by current or former employees. Under the False Claims Act, any individual can bring a claim on behalf of the government alleging that a person or entity has presented a false claim, or caused a false claim to be submitted, to the government for payment. In a qui tam suit, the government must decide whether to intervene and prosecute the case. If it declines, the individual may pursue the case alone. The person bringing a qui tam suit is entitled to a share of any recovery or settlement, up to a certain cap; the relator's share depends on the extent of the relator's involvement in the case and whether the government intervenes.

If the FDA or any other governmental agency initiates an enforcement action against us or if we are the subject of a qui tam suit and it is determined that we violated prohibitions relating to the promotion of products for unapproved uses, we could be subject to substantial civil or criminal fines or damage awards and other sanctions such as consent decrees and corporate integrity agreements pursuant to which our activities would be subject to ongoing scrutiny and monitoring to ensure compliance with applicable laws and regulations. Any such fines, awards or other sanctions would have an adverse effect on our revenue, business, financial prospects and reputation.

If approved, MRX34 or any future products may cause or contribute to adverse medical events that we are required to report to regulatory agencies, and if we fail to do so we could be subject to sanctions that would materially harm our business.

If we are successful in commercializing MRX34 or any other products, FDA and foreign regulatory agency regulations require that we report certain information about adverse medical events if those products may have caused or contributed to those adverse events. The timing of our obligation to report would be triggered by the date we become aware of the adverse event as well as the nature of the event. We may fail to report adverse events we become aware of within the

prescribed timeframe. We may also fail to appreciate that we have become aware of a reportable adverse event, especially if it is not reported to us as an adverse event or if it is an adverse event that is unexpected or removed in time from the use of our products. If we fail to comply with our reporting obligations, the FDA or a foreign regulatory agency could take action, including criminal prosecution, the imposition of civil monetary penalties, seizure of our products or delay in approval of future products. Through the first 32 months of our Phase 1 clinical trial, most of the 122 patients treated with MRX34 experienced at least one adverse event, with fever, chills, back pain, abdominal pain, nausea, diarrhea, vomiting, dehydration, anorexia, dyspnea, fatigue, headache, cough, insomnia, dysgeusia, tachycardia, anemia, neutropenia, lymphopenia, leukopenia, thrombocytopenia, elevation of liver enzymes, hyperglycemia, and hyponatremia being the most commonly reported adverse events. Two treatment-related deaths occurred during the study. Among the 47 patients in the BIW dosing cohorts, the serious adverse events determined to be related to MRX34 treatment occurring in more than one patient were fever, fatigue, dehydration and elevation of liver enzymes, each of which occurred in two patients. For the 75 patients in the QD × 5 dosing cohort, capillary leak syndrome, delirium or altered mental status, and bleeding in silent or asymptomatic HCC brain metastasis, each of which occurred in two patients, and elevation of liver enzymes, fever, and thrombocytopenia, which occurred in four patients. These adverse events associated with MRX34 are generally manageable or preventable with standard interventions or tests used by oncologists, such as administering other medications that prevent or reduce side effects, temporary slowing of infusions, delaying or stopping dosing, or using magnetic resonance imaging, or MRI, to detect silent brain metastases. Of the 42 patients with primary liver cancer treated with escalating doses of MRX34, one patient in 70 mg/ m^2 dose cohort in BIW schedule achieved confirmed partial response. Of the two acral melanoma patients enrolled in the study, one patient enrolled in the 110 mg/ m^2 dose cohort on the QD × 5 schedule achieved a confirmed partial response. Of the two metastatic renal cell carcinoma patients enrolled in the study, one patient enrolled in the 110 mg/ m^2 dose cohort on the QD × 5 schedule achieved a confirmed partial response. See “Business—MRX34: Our Lead Product Candidate” for a more detailed description of the adverse events experienced during the course of the MRX34 clinical development program.

Failure to obtain regulatory approvals in foreign jurisdictions will prevent us from marketing our product candidates internationally.

We may seek a distribution and marketing collaborator for MRX34 or other product candidates. In order to market our product candidates in the European Economic Area, or EEA (which is comprised of the 28 Member States of the EU plus Norway, Iceland and Liechtenstein), and many other foreign jurisdictions, we or any such collaborator must obtain separate regulatory approvals. More concretely, in the EEA, medicinal products can only be commercialized after obtaining a Marketing Authorization, or MA. There are two types of marketing authorizations:

- The Community MA, which is issued by the European Commission through the Centralized Procedure, based on the opinion of the Committee for Medicinal Products for Human Use of the European Medicines Agency, or EMA, and which is valid throughout the entire territory of the EEA. The Centralized Procedure is mandatory for certain types of products, such as biotechnology medicinal products, orphan medicinal products, and medicinal products indicated for the treatment of AIDS, cancer, neurodegenerative disorders, diabetes, auto-immune and viral diseases. The Centralized Procedure is optional for products containing a new active substance not yet authorized in the EEA, or for products that constitute a significant therapeutic, scientific or technical innovation or which are in the interest of public health in the EU.

- National MAs, which are issued by the competent authorities of the Member States of the EEA and only cover their respective territory, are available for products not falling within the mandatory scope of the Centralized Procedure. Where a product has already been authorized for marketing in a Member State of the EEA, this National MA can be recognized in another Member State through the Mutual Recognition Procedure. If the product has not received a National MA in any Member State at the time of application, it can be approved simultaneously in various Member States through the Decentralized Procedure.

Under these two procedures, before granting the MA, the EMA or the competent authorities of the Member States of the EEA make an assessment of the risk-benefit balance of the product on the basis of scientific criteria concerning its quality, safety and efficacy.

We have had limited interactions with foreign regulatory authorities, and the approval procedures vary among countries and can involve additional clinical testing, and the time required to obtain approval may differ from that required to obtain FDA approval. Clinical trials conducted in one country may not be accepted by regulatory authorities in other countries. Approval by the FDA does not ensure approval by regulatory authorities in other countries, and approval by one or more foreign regulatory authorities does not ensure approval by regulatory authorities in other foreign countries or by the FDA. However, a failure or delay in obtaining regulatory approval in one country may have a negative effect on the regulatory process in others. The foreign regulatory approval process may include all of the risks associated with obtaining FDA approval. We or may not obtain foreign regulatory approvals on a timely basis, if at all. We may not be able to file for regulatory approvals and even if we file, we may not receive necessary approvals to commercialize our product candidates in any market.

Healthcare reform measures could hinder or prevent our product candidates' commercial success.

In the United States, there have been, and we expect there will continue to be, a number of legislative and regulatory changes to the healthcare system that could affect our future revenues and profitability and the future revenues and profitability of our potential customers. Federal and state lawmakers regularly propose and, at times, enact legislation that results in significant changes to the healthcare system, some of which are intended to contain or reduce the costs of medical products and services. For example, in March 2010, the President signed one of the most significant healthcare reform measures in decades, the Patient Protection and Affordable Care Act, as amended by the Health Care and Education Reconciliation Act, or collectively, the Affordable Care Act. It contains a number of provisions, including those governing enrollment in federal healthcare programs, reimbursement changes and fraud and abuse measures, all of which will impact existing government healthcare programs and will result in the development of new programs. The Affordable Care Act, among other things:

- imposes a non-deductible annual fee on pharmaceutical manufacturers or importers who sell "branded prescription drugs";
- increases the minimum level of Medicaid rebates payable by manufacturers of brand-name drugs from 15.1% to 23.1%;
- addresses a new methodology by which rebates owed by manufacturers under the Medicaid Drug Rebate Program are calculated for drugs that are inhaled, infused, instilled, implanted or injected;
- requires collection of rebates for drugs paid by Medicaid managed care organizations;
- requires manufacturers to participate in a coverage gap discount program, under which they must agree to offer 50% point-of-sale discounts off negotiated prices of applicable brand drugs to eligible beneficiaries during their coverage gap period, as a condition for the manufacturer's outpatient drugs to be covered under Medicare Part D; and
- mandates a further shift in the burden of Medicaid payments to the states.

Other legislative changes have been proposed and adopted since the Affordable Care Act was enacted. On August 2, 2011, the President signed into law the Budget Control Act of 2011, which, among other things, created the Joint Select Committee on Deficit Reduction to recommend proposals in spending reductions to Congress. The Joint Select Committee did not achieve its targeted deficit reduction of at least $1.2 trillion for the years 2013 through 2021, triggering the legislation's automatic reductions to several government programs. These reductions include aggregate reductions to Medicare payments to providers of 2% per fiscal year, which went into effect on April 1, 2013 and, due to subsequent legislative amendments, will remain in effect through 2024 unless additional Congressional action is taken. On January 2, 2013, President Obama signed into law the American Taxpayer Relief Act of 2012, which, among other things, further reduced Medicare payments to several providers, including hospitals, imaging centers and cancer treatment centers, and increased the statute of limitations period for the government to recover overpayments to providers from three to five years.

There likely will continue to be legislative and regulatory proposals at the federal and state levels directed at containing or lowering the cost of health care. We cannot predict the initiatives that may be adopted in the future or their full impact. The continuing efforts of the government, insurance companies, managed care organizations and other payors of healthcare services to contain or reduce costs of health care may adversely affect:

- our ability to set a price we believe is fair for our products;
- our ability to generate revenues and achieve or maintain profitability; and
- the availability of capital.

In light of widely publicized events concerning the safety risk of certain drug products, regulatory authorities, members of Congress, the Governmental Accounting Office, medical professionals and the general public have raised concerns about potential drug safety issues. These events have resulted in the withdrawal of drug products, revisions to drug labeling that further limit use of the drug products and establishment of risk management programs that may, for instance, restrict distribution of drug products. The increased attention to drug safety issues may result in a more cautious approach by the FDA to clinical trials and the drug approval process. Data from clinical trials may receive greater scrutiny with respect to safety, which may make the FDA or other regulatory authorities more likely to terminate clinical trials before completion, or require longer or additional clinical trials that may result in substantial additional expense and a delay or failure in obtaining approval or approval for a more limited indication than originally sought. In, addition, because of the serious public health risks of high profile adverse safety events with certain products, the FDA may require, as a condition of approval, costly risk management programs which may include safety surveillance, restricted distribution and use, patient education, enhanced labeling, special packaging or labeling, expedited reporting of certain adverse events, preapproval of promotional materials and restrictions on direct-to-consumer advertising.

If we fail to comply with healthcare regulations, we could face substantial penalties and our business, results of operations and financial condition could be adversely affected.

Even though we do not and will not control referrals of healthcare services or bill directly to Medicare, Medicaid or other third-party payors, certain federal and state healthcare laws and regulations pertaining to fraud and abuse and patients' rights are and will be applicable to our business. We could be subject to healthcare fraud and abuse and patient privacy regulation by both the federal government and the states in which we conduct our business. The regulations that may affect our ability to operate include, without limitation:

- the federal Anti-Kickback Statute, which prohibits, among other things, any person from knowingly and willfully offering, soliciting, receiving or providing remuneration, directly or indirectly, to induce either the referral of an individual, for an item or service or the purchasing or ordering of a good or service, for which payment may be made under federal healthcare programs such as the Medicare and Medicaid programs. A person or entity does not need to have actual knowledge of the statute or specific intent to violate it to have committed a violation. In addition, the government may assert that a claim including items or services resulting from a violation of the federal Anti-Kickback Statute constitutes a false or fraudulent claim for purposes of the False Claims Act;
- the federal False Claims Act, which prohibits, among other things, individuals or entities from knowingly presenting, or causing to be presented, false claims, or knowingly using false statements, to obtain payment from the federal government, and which may apply to entities that provide coding and billing advice to customers;
- federal criminal laws that prohibit executing a scheme to defraud any healthcare benefit program or making false statements relating to healthcare matters. Similar to the federal Anti-Kickback Statute, a person or entity does not need to have actual knowledge of the statute or specific intent to violate it to have committed a violation;

- the federal Physician Payment Sunshine Act, which requires manufacturers of drugs, devices, biologics and medical supplies for which payment is available under Medicare, Medicaid or the Children's Health Insurance Program (with certain exceptions) to report annually to the government information related to payments or other "transfers of value" made to physicians (defined to include doctors, dentists, optometrists, podiatrists and chiropractors) and teaching hospitals, and requires applicable manufacturers and group purchasing organizations to report annually to the government ownership and investment interests held by the physicians described above and their immediate family members and payments or other "transfers of value" to such physician owners (manufacturers are required to submit reports to the government by the 90th day of each calendar year);

- the federal Health Insurance Portability and Accountability Act of 1996, as amended by the Health Information Technology for Economic and Clinical Health Act, which governs the conduct of certain electronic healthcare transactions and protects the security and privacy of protected health information; and

- state law equivalents of each of the above federal laws, such as anti-kickback and false claims laws which may apply to items or services reimbursed by any third-party payor, including commercial insurers; state laws that require pharmaceutical companies to comply with the industry's voluntary compliance guidelines and the applicable compliance guidance promulgated by the federal government or otherwise restrict payments that may be made to healthcare providers and other potential referral sources; state laws that require drug manufacturers to report information related to payments and other transfers of value to physicians and other healthcare providers or marketing expenditures; and state laws governing the privacy and security of health information in certain circumstances, many of which differ from each other in significant ways and may not have the same effect, thus complicating compliance efforts.

If our operations are found to be in violation of any of the laws described above or any other governmental regulations that apply to us, we may be subject to penalties, including civil and criminal penalties, damages, fines, exclusion from participation in government health care programs, such as Medicare and Medicaid, imprisonment, and the curtailment or restructuring of our operations, any of which could adversely affect our ability to operate our business and our financial results. Any action against us for violation of these laws, even if we successfully defend against it, could cause us to incur significant legal expenses and divert our management's attention from the operation of our business. Moreover, achieving and sustaining compliance with applicable federal and state privacy, security and fraud and abuse laws may prove costly.

Risks Related to Our Common Stock

The price of our common stock may be volatile, and you may not be able to resell your shares at or above the initial public offering price.

The trading price of our common stock could be highly volatile and could be subject to wide fluctuations in response to various factors, some of which are beyond our control. These factors include those discussed in this "Risk Factors" section of this document and others such as:

- ability to commercialize or obtain regulatory approval for our product candidates, or delays in commercializing or obtaining regulatory approval;

- results from, or any delays in, preclinical or nonclinical testing or clinical trial programs relating to our product candidates, including the Phase 1 clinical trial for MRX34;

- any need to suspend or discontinue clinical trials due to side effects or other safety risks, or any need to conduct studies on the long-term effects associated with the use of our product candidates;

- manufacturing issues related to our product candidates for clinical trials or future products for commercialization;

· commercial success and market acceptance of our product candidates following regulatory approval;

· undesirable side effects caused by product candidates after they have entered the market;

· ability to discover, develop and commercialize additional product candidates;

· announcements relating to collaborations that we may enter into with respect to the development or commercialization of our product candidates;

· announcements relating to the receipt, modification or termination of government contracts or grants;

· success of our competitors in discovering, developing or commercializing products;

· strategic transactions undertaken by us;

· additions or departures of key personnel;

· product liability claims related to our clinical trials or product candidates;

· prevailing economic conditions;

· business disruptions caused by earthquakes or other natural disasters;

· disputes concerning our intellectual property or other proprietary rights;

· FDA or other U.S. or foreign regulatory actions affecting us or our industry;

· healthcare reform measures in the United States;

· sales of our common stock by our officers, directors or significant stockholders;

· future sales or issuances of equity or debt securities by us;

· lack of an active, liquid and orderly market in our common stock;

· fluctuations in our quarterly operating results; and

· the issuance of new or changed securities analysts' reports or recommendations regarding us.

In addition, the stock markets in general, and the markets for pharmaceutical, biopharmaceutical and biotechnology stocks in particular, have experienced extreme volatility that have been often unrelated to the operating performance of the issuer. These broad market fluctuations may adversely affect the trading price or liquidity of our common stock. In the past, when the market price of a stock has been volatile, holders of that stock have sometimes instituted securities class action litigation against the issuer. If any of our stockholders were to bring such a lawsuit against us, we could incur substantial costs defending the lawsuit and the attention of our management would be diverted from the operation of our business.

Our principal stockholders and management own a significant percentage of our stock and are able to exert significant control over matters subject to stockholder approval.

Based on the beneficial ownership of our common stock as of March 31, 2016, our officers and directors, together with holders of 5% or more of our outstanding common stock and their respective affiliates, beneficially own approximately 74.9% of our common stock. Accordingly, these stockholders have significant influence over the outcome

of corporate actions requiring stockholder approval, including the election of directors, any merger, consolidation or sale of all or substantially all of our assets or any other significant corporate transaction. The interests of these stockholders may not be the same as or may even conflict with your interests. For example, these stockholders could delay or prevent a change of control of our company, even if such a change of control would benefit our other stockholders, which could deprive our stockholders of an opportunity to receive a premium for their common stock as part of a sale of our company or our assets and might affect the prevailing market price of our common stock. The significant concentration of stock ownership may adversely affect the trading price of our common stock due to investors' perception that conflicts of interest may exist or arise.

We are an "emerging growth company" and we cannot be certain if the reduced disclosure requirements applicable to emerging growth companies will make our common stock less attractive to investors.

We are an "emerging growth company," as defined in the JOBS Act, and may take advantage of certain exemptions from various reporting requirements that are applicable to other public companies that are not "emerging growth companies" including not being required to comply with the auditor attestation requirements of Section 404 of the Sarbanes-Oxley Act, reduced disclosure obligations regarding executive compensation in our periodic reports and proxy statements and exemptions from the requirements of holding a nonbinding advisory vote on executive compensation and shareholder approval of any golden parachute payments not previously approved. We cannot predict if investors will find our common stock less attractive because we may rely on these exemptions. If some investors find our common stock less attractive as a result, there may be a less active trading market for our common stock and our stock price may be more volatile.

In addition, Section 102 of the JOBS Act also provides that an "emerging growth company" can take advantage of the extended transition period provided in Section 7(a)(2)(B) of the Securities Act of 1933, as amended, or the Securities Act, for complying with new or revised accounting standards. An "emerging growth company" can therefore delay the adoption of certain accounting standards until those standards would otherwise apply to private companies. However, we are choosing to "opt out" of such extended transition period, and as a result, we will comply with new or revised accounting standards on the relevant dates on which adoption of such standards is required for non-emerging growth companies. Section 107 of the JOBS Act provides that our decision to opt out of the extended transition period for complying with new or revised accounting standards is irrevocable.

Future sales of our common stock or securities convertible or exchangeable for our common stock may depress our stock price.

If our existing stockholders or holders of our options sell, or indicate an intention to sell, substantial amounts of our common stock in the public market after the lock-up and legal restrictions on resale discussed in this periodic report lapse, the trading price of our common stock could decline. The perception in the market that these sales may occur could also cause the trading price of our common stock to decline. Of the 20,830,555 shares of common stock outstanding at March 31, 2016, the 6,954,962 shares of common stock sold by us in the IPO, plus up to 13,875,593 shares subject to lock-up agreements in connection with the IPO that expired on March 28, 2016, are currently freely tradable without restriction, unless held by our affiliates, in the public market.

In addition, based on the number of shares subject to outstanding awards under our 2008 Long Term Incentive Plan, or 2008 Stock Plan, as of March 31, 2016, and including the initial reserves under our 2015 Equity Incentive Award Plan, or 2015 Plan, and Employee Stock Purchase Plan, or ESPP, approximately 3.9 million shares of common stock that are either subject to outstanding options, outstanding but subject to vesting, or reserved for future issuance under the 2008 Stock Plan, 2015 Plan or ESPP will become eligible for sale in the public market to the extent permitted by the provisions of various vesting schedules, the lock-up agreements and Rule 144 and Rule 701 under the Securities Act. We also filed a registration statement permitting certain shares of common stock issued in the future pursuant to the 2008 Plan, 2015 Plan and ESPP to be freely resold by plan participants in the public market, subject to the lock-up agreements, applicable vesting schedules and, for shares held by directors, executive officers and other affiliates, volume limitations under Rule 144 under the Securities Act. The 2015 Plan and ESPP also contain provisions for the annual increase of the number of shares reserved for issuance under such plans, which shares we also intend to register. If the

shares we may issue from time to time under the 2008 Stock Plan, 2015 Plan or ESPP are sold, or if it is perceived that they will be sold, by the award recipient in the public market, the trading price of our common stock could decline.

Certain holders of approximately 13.9 million shares of our common stock at December 31, 2015 are entitled to rights with respect to the registration of their shares under the Securities Act, subject to the lock-up agreements described above. Registration of these shares under the Securities Act would result in the shares becoming freely tradable without restriction under the Securities Act, except for shares purchased by affiliates. Sales of such shares could also cause the trading price of our common stock to decline.

An active, liquid and orderly market for our common stock may not develop.

Prior to our IPO in October 2015, there had been no public market for our common stock, and an active public market for our shares may not develop or be sustained. Further, certain of our existing institutional investors, including investors affiliated with certain of our directors, purchased approximately 2.4 million shares of common stock in our IPO and consequently fewer shares may be actively traded in the public market because these stockholders are restricted from selling the shares by restrictions under applicable securities laws and the lock-up agreements entered into in connection with our IPO, which would reduce the liquidity of the market for our common stock. The lack of an active market may impair our stockholders' ability to sell their shares at the time they wish to sell them or at a price that they consider reasonable or may result in volatility in our stock price. An inactive market may also impair our ability to raise capital by selling shares and may impair our ability to acquire other businesses or technologies or in-license new product candidates using our shares as consideration.

Our quarterly operating results may fluctuate significantly or may fall below the expectations of investors or securities analysts, each of which may cause our stock price to fluctuate or decline.

We expect our operating results to be subject to quarterly fluctuations. Our net loss and other operating results will be affected by numerous factors, including:

- variations in the level of expenses related to our product candidates or future development programs;
- if MRX34 or any other product candidate receives regulatory approval, the level of underlying demand for these product candidates;
- addition or termination of clinical trials or funding support;
- receipt, modification or termination of government contracts or grants, and the timing of payments we receive under these arrangements;
- our execution of any collaborative, licensing or similar arrangements, and the timing of payments we may make or receive under these arrangements;
- any intellectual property infringement lawsuit or opposition, interference or cancellation proceeding in which we may become involved; and
- regulatory developments affecting our product candidates or those of our competitors.

If our quarterly operating results fall below the expectations of investors or securities analysts, the price of our common stock could decline substantially. Furthermore, any quarterly fluctuations in our operating results may, in turn, cause the price of our stock to fluctuate substantially. We believe that quarterly comparisons of our financial results are not necessarily meaningful and should not be relied upon as an indication of our future performance.

We will have broad discretion to determine how to use the net proceeds of our IPO and the concurrent private placement and may not use them effectively.

Our management has broad discretion over the use of the net proceeds from our IPO and the concurrent private placement described in the Prospectus. Because of the number and variability of factors that will determine our use of such proceeds, you may not agree with how we allocate or spend the proceeds from our IPO and the concurrent private placement. We may pursue collaborations or clinical trials that do not result in an increase in the market value of our common shares and that may increase our losses. Our failure to allocate and spend the net proceeds from our IPO and the concurrent private placement effectively would have a material adverse effect on our business, financial condition and results of operations. Until the net proceeds are used, they may be placed in investments that do not produce significant investment returns or that may lose value.

Provisions of our charter documents or Delaware law could delay or prevent an acquisition of our company, even if the acquisition would be beneficial to our stockholders, and could make it more difficult for you to change management.

Provisions in our amended and restated certificate of incorporation and our amended and restated bylaws may discourage, delay or prevent a merger, acquisition or other change in control that stockholders may consider favorable, including transactions in which stockholders might otherwise receive a premium for their shares. In addition, these provisions may frustrate or prevent any attempt by our stockholders to replace or remove our current management by making it more difficult to replace or remove our board of directors. These provisions include:

- a classified board of directors so that not all directors are elected at one time;
- a prohibition on stockholder action through written consent;
- no cumulative voting in the election of directors;
- the exclusive right of our board of directors to elect a director to fill a vacancy created by the expansion of the board of directors or the resignation, death or removal of a director;
- a requirement that special meetings of stockholders be called only by the board of directors, the chairman of the board of directors, the chief executive officer or, in the absence of a chief executive officer, the president;
- an advance notice requirement for stockholder proposals and nominations;
- the authority of our board of directors to issue preferred stock with such terms as our board of directors may determine; and
- a requirement of approval of not less than $66^{2}/_{3}\%$ of all outstanding shares of our capital stock entitled to vote to amend any bylaws by stockholder action, or to amend specific provisions of our certificate of incorporation.

In addition, Delaware law prohibits a publicly held Delaware corporation from engaging in a business combination with an interested stockholder, generally a person who, together with its affiliates, owns or within the last three years has owned 15% or more of our voting stock, for a period of three years after the date of the transaction in which the person became an interested stockholder, unless the business combination is approved in a prescribed manner. Accordingly, Delaware law may discourage, delay or prevent a change in control of our company. Furthermore, our amended and restated certificate of incorporation will specify that the Court of Chancery of the State of Delaware will be the sole and exclusive forum for most legal actions involving actions brought against us by stockholders. We believe this provision benefits us by providing increased consistency in the application of Delaware law by chancellors particularly experienced in resolving corporate disputes, efficient administration of cases on a more expedited schedule relative to other forums and protection against the burdens of multi-forum litigation. However, the provision may have the effect of

discouraging lawsuits against our directors and officers. The enforceability of similar choice of forum provisions in other companies' certificates of incorporation has been challenged in legal proceedings, and it is possible that, in connection with any applicable action brought against us, a court could find the choice of forum provisions contained in our amended and restated certificate of incorporation to be inapplicable or unenforceable in such action.

Provisions in our charter and other provisions of Delaware law could limit the price that investors are willing to pay in the future for shares of our common stock.

Our employment agreements with our officers may require us to pay severance benefits to any of those persons who are terminated in connection with a change of control of us, which could harm our business, financial condition or results of operations.

Our officers are parties to employment agreements providing for aggregate cash payments of up to approximately $2.8 million at March 31, 2016 for severance and other benefits in the event of a termination of employment in connection with a change of control of us. The payment of these severance benefits could harm our business, financial condition and results of operations. In addition, these potential severance payments may discourage or prevent third parties from seeking a business combination with us.

We do not anticipate paying any cash dividends on our common stock in the foreseeable future; therefore, capital appreciation, if any, of our common stock will be your sole source of gain for the foreseeable future.

We have never declared or paid cash dividends on our common stock. We do not anticipate paying any cash dividends on our common stock in the foreseeable future. We currently intend to retain all available funds and any future earnings to fund the development and growth of our business. In addition, the terms of any future debt financing arrangement may contain terms prohibiting or limiting the amount of dividends that may be declared or paid on our common stock. As a result, capital appreciation, if any, of our common stock will be your sole source of gain for the foreseeable future.

If securities or industry analysts do not publish research, or publish inaccurate or unfavorable research, about our business, our stock price and trading volume could decline.

The trading market for our common stock will depend, in part, on the research and reports that securities or industry analysts publish about us or our business. Securities and industry analysts do not currently, and may never, publish research on our company. If no securities or industry analysts commence coverage of our company, the trading price for our common stock would likely be negatively impacted. In the event securities or industry analysts initiate coverage, if one or more of the analysts who cover us downgrade our common stock or publish inaccurate or unfavorable research about our business, our stock price would likely decline. In addition, if our operating results fail to meet the forecast of analysts, our stock price would likely decline. If one or more of these analysts cease coverage of our company or fail to publish reports on us regularly, demand for our common stock could decrease, which might cause our stock price and trading volume to decline.

Changes in, or interpretations of, accounting rules and regulations could result in unfavorable accounting charges or require us to change our compensation policies.

Accounting methods and policies for biopharmaceutical companies, including policies governing revenue recognition, research and development and related expenses and accounting for stock-based compensation, are subject to further review, interpretation and guidance from relevant accounting authorities, including the SEC. Changes to, or interpretations of, accounting methods or policies may require us to reclassify, restate or otherwise change or revise our financial statements, including those contained in this periodic report.

Item 2. Unregistered Sales of Equity Securities and Use of Proceeds

None.

Item 3. Defaults Upon Senior Securities.

None.

Item 4. Mine Safety Disclosures.

Not applicable.

Item 5. Other Information.

None.

Item 6. Exhibits

		Incorporated by Reference			
Exhibit Number	**Description of Document**	**Form**	**Date**	**Number**	**Filed Herewith**
3.1	Amended and Restated Certificate of Incorporation.	8-K	10/06/2015	3.1	
3.2	Amended and Restated Bylaws.	8-K	10/06/2015	3.2	
4.1	Reference is made to Exhibits 3.1 through 3.2.				
4.2	Form of Common Stock Certificate.	S-1/A	09/18/2015	4.2	
31.1	Certification of Chief Executive Officer required by Rule 13a-14(a) or Rule 15d-14(a).				X
31.2	Certification of Chief Financial Officer required by Rule 13a-14(a) or Rule 15d-14(a).				X
32.1*	Certification required by Rule 13a-14(b) or Rule 15d-14(b) and Section 1350 of Chapter 63 of Title 18 of the United States Code (18 U.S.C. §1350)				X
101.INS	XBRL Instance Document				X
101.SCH	XBRL Taxonomy Extension Schema Document				X
101.CAL	XBRL Taxonomy Extension Calculation Linkbase Document				X
101.DEF	XBRL Taxonomy Extension Definition Linkbase Document				X
101.LAB	XBRL Taxonomy Extension Label Linkbase Document				X
101.PRE	XBRL Taxonomy Extension Presentation Linkbase Document				X

* The certification attached as Exhibit 32.1 that accompanies this Quarterly Report on Form 10-Q is not deemed filed with the SEC and is not to be incorporated by reference into any filing of Mirna Therapeutics, Inc. under the Securities Act of 1933, as amended, or the Securities Exchange Act of 1934, as amended, whether made before or after the date of this Quarterly Report on Form 10-Q, irrespective of any general incorporation language contained in such filing.

SIGNATURES

Pursuant to the requirements of the Securities Exchange Act of 1934, as amended, the registrant has duly caused this report to be signed on its behalf by the undersigned thereunto duly authorized.

MIRNA THERAPEUTICS, INC.
(Registrant)

Date: May 12, 2016

/s/ Paul Lammers
Paul Lammers, M.D., M.Sc.
Chief Executive Officer
(Principal Executive Officer)

Date: May 12, 2016

/s/ Alan Fuhrman
Alan Fuhrman
Chief Financial Officer
(Principal Financial Officer)

Exhibit 31.1

CERTIFICATION OF THE CHIEF EXECUTIVE OFFICER

PURSUANT TO

SECURITIES EXCHANGE ACT RULES 13A-14(A) AND 15D-14(A)

I, Paul Lammers, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q of Mirna Therapeutics, Inc.;

2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;

3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;

4. The registrant's other certifying officer(s) and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) for the registrant and have:

   (a) Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;

   (b) Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and

   (c) Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and

5. The registrant's other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):

   (a) All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and

   (b) Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: May 12, 2016

/s/ PAUL LAMMERS
Paul Lammers, M.D., M.Sc.
Chief Executive Officer
(Principal Executive Officer)

Exhibit 31.2

CERTIFICATION OF THE CHIEF FINANCIAL OFFICER

PURSUANT TO

SECURITIES EXCHANGE ACT RULES 13A-14(A) AND 15D-14(A)

I, Alan Fuhrman, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q of Mirna Therapeutics, Inc.;

2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;

3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;

4. The registrant's other certifying officer(s) and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) for the registrant and have:

   (a) Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;

   (b) Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and

   (c) Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and

5. The registrant's other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):

   (a) All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and

   (b) Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: May 12, 2016

/s/ ALAN FUHRMAN
Alan Fuhrman
Chief Financial Officer
(Principal Financial Officer)

Exhibit 32.1

CERTIFICATION PURSUANT TO
18 U.S.C. SECTION 1350,
AS ADOPTED PURSUANT TO
SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002

In connection with the Quarterly Report of Mirna Therapeutics, Inc. (the "Company") on Form 10-Q for the fiscal quarter ended March 31, 2016, as filed with the Securities and Exchange Commission (the "Report"), Paul Lammers, Chief Executive Officer of the Company, and Alan Fuhrman, Chief Financial Officer of the Company, respectively, do each hereby certify, pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, that:

- The Report fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934; and
- The information in the Report fairly presents, in all material respects, the financial condition and results of operations of the Company.

Date: May 12, 2016

/s/ PAUL LAMMERS
Paul Lammers, M.D., M.Sc.
Chief Executive Officer
(Principal Executive Officer)

/s/ ALAN FUHRMAN
Alan Fuhrman
Chief Financial Officer
(Principal Financial Officer)